Ocular Therapeutix™ Announces Plans to Accelerate NDA Submission Timeline for AXPAXLI™ in Wet AMD

December 8, 2025

Ocular intends to submit AXPAXLI New Drug Application (NDA) for wet AMD shortly after year one data from SOL-1, if positive

SOL-1 topline data remain on track for 1Q 2026

Ocular plans to leverage the 505(b)(2) regulatory pathway for new drug approvals which has the potential to shorten the review timeline for AXPAXLI

If approved, AXPAXLI could be the first TKI to be commercialized in wet AMD, with a potential superiority label and best-in-class durability

BEDFORD, Mass., Dec. 08, 2025 (GLOBE NEWSWIRE) -- Ocular Therapeutix, Inc. (NASDAQ: OCUL, "Ocular"), an integrated biopharmaceutical company committed to redefining the retina experience, announced that following recent public statements from U.S. Food and Drug Administration (FDA) leadership and other recent interactions with the FDA's Division of Ophthalmology, the Company now intends to submit a New Drug Application (NDA) for AXPAXLI™ (also known as OTX-TKI) for the treatment of wet age-related macular degeneration (wet AMD) following year one data, if positive, from its ongoing SOL-1 Phase 3 clinical trial for which data are on track for the first quarter of 2026.

"At Ocular Therapeutix, we have always been courageous, opportunistic, and bold in our efforts to redefine retina. Based on recent developments, we now intend to submit our NDA for AXPAXLI in wet AMD shortly after SOL-1 year one data, assuming positive results. SOL-1 is the only ongoing Phase 3 retina trial currently being conducted under a Special Protocol Assessment (SPA) agreement. It is also the only current wet AMD trial exploring superiority compared to a single injection of aflibercept (2 mg) and the only wet AMD registrational trial that we are aware of that is being run completely in alignment with the FDA's draft guidance and feedback. This triad of factors helps us make a compelling case for a truly differentiated NDA for AXPAXLI in the treatment of wet AMD," said **Pravin U. Dugel, MD, Executive Chairman, President and Chief Executive Officer of Ocular Therapeutix**. "Recent FDA leadership comments on the potential for a single registrational trial for new product candidates stressed the importance that these studies be well powered, and well controlled. Being a superiority trial, SOL-1 is substantially powered compared to non-inferiority trials. Moreover, SOL-1 is well controlled, as the active and control arms have the same dosing cadence and do not use sham injections for masking, as per the FDA's draft guidance and feedback. Since axitinib, the active component of AXPAXLI, is already approved in non-ophthalmic indications, we intend to leverage the 505(b)(2) pathway, which we believe has the potential to further accelerate our review timeline. With AXPAXLI's potential to redefine treatment as the first TKI to market with differentiated and potentially best-in-class durability, we believe AXPAXLI can meaningfully and immediately change the standard of care."

The FDA has historically required two adequate and well-controlled clinical trials to demonstrate the safety and efficacy of ophthalmic product candidates, particularly for larger indications of use such as wet AMD. Recent statements from FDA leadership indicate that the Agency is potentially moving to requiring only a single registrational trial for approval, as long as the trial is adequately powered and controlled. Based on Ocular's SPA agreement for SOL-1, along with the trial's superiority design, the Company plans to work with the FDA to submit its NDA for AXPAXLI in wet AMD following year one results from SOL-1. The Company expects that additional data from the continuation of SOL-1 in year two, SOL-R, and SOL-X will help clinicians and payors appreciate the anticipated benefits of AXPAXLI's efficacy, safety, and durability, allowing them to seamlessly adopt AXPAXLI into clinical practice. The Company will engage further with the FDA regarding next steps on the regulatory pathway for AXPAXLI and will provide updates as appropriate.

"Wet AMD remains one of the most challenging diseases we treat because the real-world burden of frequent injections is unsustainable for many, if not most, patients. We urgently need a therapy that delivers meaningfully better durability without adding complexity to clinical practice," said **Jeffrey S. Heier, MD, Chief Scientific Officer of Ocular Therapeutix**. "AXPAXLI has the potential to transform how we manage wet AMD, with the goal of delivering truly differentiated durability of up to 12 months, with potential for better treatment adherence in the short term and improved visual outcomes over the long-term. Most importantly, retina specialists would not need to change anything about how they treat patients today – no surgery, no concomitant steroids, no new procedures. It is the same intravitreal injection procedure we use every day, but with the potential for the therapy to last far longer, with possibly better long-term outcomes. That simplicity is key to broad and rapid adoption. If successful, AXPAXLI could significantly reduce treatment discontinuation, improve long-term outcomes, and redefine the wet AMD treatment landscape."

Wet AMD remains a leading cause of blindness worldwide, affecting approximately 14.5 million individuals globally and 1.8 million in the United States alone. Despite advances in anti-VEGF therapy, many patients require frequent injections to maintain vision,

and up to 40% discontinue treatment within the first year, leading to disease progression and vision loss. AXPAXLI is being developed to address this unmet need with the potential to extend dosing intervals to every 6 to 12 months and potentially provide superior and sustainable long-term visual outcomes.

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, and other retinal diseases.

About the SOL-1 Study
The registrational Phase 3 SOL-1 trial (NCT06223958) is designed to evaluate the safety and efficacy of AXPAXLI in a multi-center, double-masked, randomized (1:1), parallel group study that involves more than 100 clinical trial sites located in the U.S. and Argentina. In December 2024, the trial completed randomization of 344 evaluable treatment-naïve subjects with a diagnosis of wet AMD in the study eye.

The superiority study has an eight-week loading segment prior to randomization. During the loading segment, subjects who have 20/80 vision or better and a central subfield thickness (CSFT) of ≤500 µm receive two doses of aflibercept (2 mg) at Week -8 and Week -4. Subjects who achieve best corrected visual acuity (BCVA) of 20/20 at Day 1 or gain at least 10 early treatment diabetic retinopathy study (ETDRS) letters at Day 1 along with a CSFT of ≤350 µm are then randomized to receive a single dose of AXPAXLI or a single dose of aflibercept (2 mg). At Week 52 and at Week 76, all subjects are re-dosed with their respective initial treatment of AXPAXLI or aflibercept (2 mg). Subjects will be followed for safety until the end of Year 2. Throughout the study, subjects are assessed monthly. Trial subjects and designated study personnel will remain masked through the end of Year 2. The clinical trial protocol requires that, during the study, subjects in either arm meeting pre-specified rescue criteria will receive a supplemental dose of aflibercept (2 mg).

The primary endpoint of SOL-1 is the proportion of subjects who maintain visual acuity, defined as a loss of <15 ETDRS letters of BCVA, at Week 36. Subjects will continue to be evaluated for durability up to Week 52. The study is being conducted under a Special Protocol Assessment (SPA) agreement with the FDA.

About Wet AMD
Wet age-related macular degeneration (wet AMD) is a leading cause of severe, irreversible vision loss affecting approximately 14.5 million individuals globally and 1.8 million in the United States alone. Wet AMD causes vision loss due to abnormal new blood vessel growth and hyperpermeability and associated retinal vascularity in the macula, which is primarily stimulated by local upregulation of vascular endothelial growth factor (VEGF). Without prompt and continuous treatment to control this exudative activity, patients develop irreversible vision loss. With proper treatment, patients may maintain visual function for a period of time and may temporarily regain lost vision. Challenges with current therapies include pulsatile, repeated intraocular injections, treatment-related adverse events and up to 40% patient discontinuation within one year of initiating treatment with continued disease progression. Taken together, these factors lead to undertreatment and a lack of long-term vision improvement for patients.

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) and non-proliferative diabetic retinopathy (NPDR).

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 has completed a Phase 2 clinical trial for the treatment of open-angle glaucoma or ocular hypertension. Ocular is currently evaluating next steps for the OTX-TIC program.

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

DEXTENZA® is a registered trademark of Ocular Therapeutix, Inc. The Ocular Therapeutix logo, AXPAXLI™, ELUTYX™, and Ocular Therapeutix™ are trademarks of Ocular Therapeutix, Inc.

Forward-Looking Statements
This press release contains forward-looking statements of the Company regarding its future expectations, plans, and prospects; statements regarding the development and regulatory status of the Company's product candidate AXPAXLI (also known as OTX-TKI), including the Company's intentions, assuming the data are positive, to submit a new drug application for AXPAXLI based on year one data from the Company's SOL-1 Phase 3 clinical trial of AXPAXLI for the treatment of wet age-related macular degeneration; statements regarding the timing of the availability of data from the SOL-1 trial; 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, all of which 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, uncertainties regarding the initiation, design, timing, conduct and outcomes of ongoing and planned clinical trials, including the Company's SOL-1 trial, SOL-R trial, planned SOL-X trial, planned HELIOS-2 trial, and HELIOS-3 trial; the risk that the U.S. Food and Drug Administration, or FDA, will not agree with the Company's interpretation of the written agreements under the Special Protocol Assessments for AXPAXLI, including for the SOL-1 trial; uncertainty as to whether the FDA will accept a new drug application for AXPAXLI on the basis of a single pivotal clinical trial, even if SOL-1 data are positive; uncertainty as to the minimum clinical data required to demonstrate the safety of a proposed product candidate such as AXPAXLI, even if the FDA recognizes that only one pivotal clinical trial may be required to demonstrate efficacy; the risk that even though the FDA has agreed with the overall design of the SOL-1 trial, the FDA may not find that the data generated by the trial and submitted by the Company, even if positive, are sufficient to demonstrate the safety and efficacy of AXPAXLI to the degree necessary to support marketing approval for wet age-related macular degeneration; the risk that the FDA might not agree to the Company's design, protocol, and statistical analysis plan of any of its clinical trials for which the Company has not obtained a Special Protocol Assessment; the risk that the Company and the FDA may not agree on the registrational pathway for any of its product candidates, including AXPAXLI; uncertainty as to whether the Company will be able to timely satisfy the FDA's other requirements for regulatory approval of AXPAXLI, including the FDA's Chemistry, Manufacturing and Control's requirements, even if the Company can satisfy the FDA's clinical requirements to demonstrate safety and efficacy; uncertainty as to what restrictions, if any, may be imposed on the label for AXPAXLI, if approved, pending the receipt of additional clinical data or otherwise; 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 or SOL-R 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 the Company's ability to retain regulatory approval of any product or product candidate that receives regulatory approval; uncertainty as to whether data from the Company's SOL-X trial 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