



Ophthotech Completes Patient Recruitment As Planned for its Phase 2b Clinical Trial of Zimura® Monotherapy for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

October 4, 2018

- Initial Top-line Zimura Geographic Atrophy Data Expected in the Fourth Quarter of 2019 -

- Zimura Wet AMD Data Continues on Track for Initial Top-line Data Before the End of 2018 -

NEW YORK--(BUSINESS WIRE)--Oct. 4, 2018-- Ophthotech Corporation (NASDAQ:OPHT) announced completion of patient recruitment for its Phase 2b clinical trial of Zimura® (avacincaptad pegol), the Company's complement factor C5 inhibitor, monotherapy in patients with geographic atrophy secondary to dry age-related macular degeneration (AMD). Complement factor C5 is a central component of the complement cascade and is believed to be involved in the development and progression of AMD. A total of 286 patients have been enrolled into this randomized, double-masked, sham controlled multi-center clinical trial. This clinical trial is designed to assess the safety and efficacy of various Zimura dosing regimens over 12 months. Patients will continue to be treated and monitored until month 18.

"We are grateful to our principal investigators and their dedicated clinical staffs for their interest and support of our Zimura program, enabling on time completion of recruitment for our clinical trial in patients with geographic atrophy secondary to dry AMD," stated Kourous A. Rezaei, M.D., Chief Medical Officer of Ophthotech. "Recent clinical data together with the pre-clinical scientific evidence implicating complement in various retinal diseases further invigorates our enthusiasm for the therapeutic potential of Zimura. We look forward to initial top-line data from this clinical trial, which is expected to be available during the fourth quarter of 2019."

The Company also expects initial top-line data for its open-label Phase 2a clinical trial of Zimura combination therapy with the anti-vascular endothelial growth factor (anti-VEGF) agent Lucentis® (ranibizumab) 0.5 mg in treatment-naïve patients with wet AMD before the end of this year. A total of 64 patients have been enrolled into this randomized, dose-ranging, open-label, uncontrolled, multi-center trial. This trial is designed to assess the safety of different dosages of Zimura in combination with Lucentis and to detect a potential efficacy signal at month 6. Following the completion of this trial, clinical data will be analyzed to assess whether to proceed to a randomized, sham-controlled clinical trial of Zimura combination therapy with anti-VEGF in wet AMD.

Further, Zimura is being evaluated in a Phase 2b clinical trial in patients with autosomal recessive Stargardt disease (STGD1) and clinical trial sites are currently enrolling patients. Patients interested in additional information about this study can call toll-free 1-833-STGD1-OP (1-833-784-3167). More information on these clinical trials is provided at www.clinicaltrials.gov.

Dry AMD / Geographic Atrophy

Dry AMD is a significant cause of moderate and severe loss of central vision, affecting both eyes in the majority of patients. Although dry AMD is the most common form of AMD, there are no U.S. Food and Drug Administration or European Medicines Agency approved therapies to treat this condition. In dry AMD, thinning of the retinal pigment epithelial (RPE) cells in the macula develops, along with other age-related changes to the adjacent retinal and choroidal tissue layers. Geographic atrophy, the end stage of dry AMD, is a disease characterized by degeneration of retinal tissue leading to further loss of vision.

About Zimura

Zimura is designed to target and inhibit the complement protein C5. Zimura is believed to bind to C5 and inhibit it from cleaving into the terminal fragments, C5a and C5b. By inhibiting the formation of complement system terminal fragments, Zimura may decrease the activation of inflammasomes and decrease the formation of membrane attack complex (MAC), thereby potentially avoiding or slowing the degeneration of RPE cells and providing the rationale as a potential therapy for the retinal conditions that are being targeted.

About Ophthotech Corporation

Ophthotech is a science-driven biopharmaceutical company specializing in the development of novel therapies to treat ophthalmic diseases, with a focus on age-related and orphan retinal diseases. For more information, please visit www.opthotech.com.

Forward-looking Statements

Any statements in this press release about Ophthotech's future expectations, plans and prospects constitute forward-looking statements for purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995. Forward-looking statements include any statements about Ophthotech's strategy, future operations and future expectations and plans and prospects for Ophthotech, and any 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. In this press release, Ophthotech's forward-looking statements include statements about the implementation of its strategic plan, the timing, progress and results of clinical trials and other research and development activities and the potential utility of its product candidates. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech'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, those related to the initiation and the conduct and design of research programs and clinical trials, availability of data from these programs, expectations for regulatory matters, need for additional financing and other factors discussed in the "Risk Factors" section contained in the quarterly and annual reports that Ophthotech files with the Securities and Exchange Commission. Any forward-looking statements represent Ophthotech's views only as of the date of this press release. Ophthotech anticipates that subsequent events and developments will cause its views to change. While Ophthotech may elect to update these forward-looking statements at some point in the future, Ophthotech specifically disclaims any obligation to do so except as required by law.

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