



## Ophthotech Provides Update on Zimura® Complement Programs for Treatment of Eye Diseases

- *Company Initiates Phase 2a Clinical Trial in Patients with Wet Age-related Macular Degeneration* –
- *Clinical Trials in Stargardt Disease and Idiopathic Polypoidal Choroidal Vasculopathy on Schedule to Initiate Before the End of the Year* –
- *Company Modifies Plan for Study in Geographic Atrophy, a form of Dry Age-related Macular Degeneration* –

**New York, NY – September 19, 2017** – Ophthotech Corporation (NASDAQ: OPHT) announced today the initiation of an open-label Phase 2a clinical trial of Zimura® (avacincaptad pegol), the Company’s complement factor C5 inhibitor, in patients with wet age-related macular degeneration (AMD). Zimura will be administered in combination with Lucentis® (ranibizumab), an anti-vascular endothelial growth factor (anti-VEGF), in patients with wet AMD who have not been previously treated with anti-VEGF drugs. A range of Zimura dosing regimens will be assessed. Clinical trial sites have been identified and activated.

“We believe that supplementing anti-VEGF therapy with a complement inhibitor such as Zimura may have the potential to further enhance the efficacy of anti-VEGF monotherapy in wet AMD,” stated Kourous A. Rezaei, M.D., Senior Vice President of Medical Strategy. “Our earlier Phase 1/2a clinical trial assessing Zimura in combination with Lucentis, while small and uncontrolled, showed intriguing results. A recent peer-reviewed publication from the *Journal of Clinical Investigation from Scripps Research Institute* provided further support that anti-VEGF therapy upregulates complement activation and therefore that complement inhibition during anti-VEGF therapy may have therapeutic merit. These findings establish a foundation to pursue further development of Zimura in wet AMD.”

“We are excited to move the Company forward with opportunities consistent with our overall strategy to develop Zimura for both orphan diseases and larger indications in the back of the eye, such as age-related retinal diseases,” stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech.

The Company also announced that it remains on track to initiate two additional Zimura clinical trials before the end of the year. The Company’s strategy in orphan indications will be led by a randomized, controlled clinical trial to assess Zimura monotherapy in Stargardt disease, a devastating orphan retinal disease-causing vision loss during childhood or adolescence. The other trial will be an open-label Phase 2a clinical trial evaluating Zimura in combination with anti-VEGF therapy for idiopathic polypoidal choroidal vasculopathy (IPCV), an age-related eye disease. Ophthotech is also planning a Phase 2a clinical trial of Zimura monotherapy for intermediate/posterior non-infectious uveitis, an orphan inflammatory disease of the back of the eye. This trial is planned to initiate in 2018.

The Company is in the process of modifying its dry AMD program. Following the recent announcements of competitors’ conflicting topline results from two clinical trials assessing the role of two different types of complement inhibitors, one blocking the alternative complement pathway (did not meet primary endpoint) and the other blocking all three complement pathways (met primary endpoint), in the treatment of geographic atrophy, a dry form of AMD, Ophthotech

has decided to modify its ongoing Phase 2/3 clinical trial of Zimura monotherapy (which blocks all three complement pathways) in geographic atrophy. Ophthotech had originally planned to enroll 300 patients in an initial stage of the ongoing trial, with an interim analysis scheduled for the 18-month time point, and to potentially enroll up to an additional 600 patients thereafter. The trial will be modified to accelerate the anticipated timeline to obtain topline data by reducing the number of patients, shortening the time point for attaining the primary efficacy endpoint and thereby reducing the cost to complete the study. The modified study design will incorporate patients already enrolled in the study.

### **About Zimura**

Zimura is designed to target and inhibit the complement protein C5, a central component of the complement cascade. Inhibition of C5 in the complement cascade prevents the formation of key terminal fragments (C5a and C5b-9) regardless of which of the three complement pathways (classical, lectin or alternative) induced their generation. C5b-9 is involved in the formation of the membrane attack complex (MAC: C5b-9), which can cause cell death through disruption of the cell membrane. By inhibiting the terminal steps of complement activation at the level of C5, we believe a therapeutic benefit may be achieved.

### **About Ophthotech Corporation**

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics for diseases of the eye. For more information, please visit [www.ophthotech.com](http://www.ophthotech.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, Ophthotech's projected use of cash and cash balances, the timing, progress and results of clinical trials and other development activities, and the potential utility or commercialization of any of Ophthotech's product candidates. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech'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, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory matters 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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