



Ophthotech Announces First Patient Enrolled in the Phase 2b Clinical Trial of Zimura®, Complement C5 Inhibitor, for the Treatment of Autosomal Recessive Stargardt Disease (STGD1)

January 16, 2018

- One of the Largest Interventional Clinical Trials for the Treatment of STGD1, an Inherited Orphan Retinal Disease –

- Zimura Is Also Being Investigated in Ongoing Clinical Trials for the Treatment of Dry and Wet Age-related Macular Degeneration (AMD) and Idiopathic Polypoidal Choroidal Vasculopathy (PCV) -

NEW YORK--(BUSINESS WIRE)--Jan. 16, 2018-- Ophthotech Corporation (NASDAQ:OPHT) announced today that the first patient has been enrolled in the Phase 2b randomized, double-masked, sham-controlled clinical trial assessing the efficacy and safety of Zimura® (avacincaptad pegol), complement C5 inhibitor, in patients with autosomal recessive Stargardt disease (STGD1). STGD1 is an inherited orphan retinal disease causing vision loss during childhood or adolescence. Currently there are no FDA or EMA approved treatment options available for patients with STGD1. Over 30 sites have been identified for this clinical trial, making it one of the largest interventional clinical trials for STGD1 to date. The scientific details of the Stargardt clinical trial will be presented at the 2018 Annual Meeting of the Association for Research in Vision and Ophthalmology (ARVO) in Honolulu, Hawaii.

"We are excited to focus and accelerate the development of Zimura in Stargardt disease," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "We look forward to 2018 with our ongoing clinical programs in dry AMD, wet AMD and PCV. We continue our search for new opportunities that are in sync with our commitment to develop novel therapeutics for age-related and orphan diseases in the back of the eye."

"Recent scientific literature emphasizes the role of complement in Stargardt disease, supporting our strategy for the development of Zimura in this devastating orphan retinal condition," stated Kourous A. Rezaei, M.D., Senior Vice President and Chief Medical Officer of Ophthotech. "Our work with the highly-distinguished organization, Foundation Fighting Blindness, provided us access to publicly available data from ProgStar, the largest natural history study in autosomal recessive Stargardt disease to date, playing an integral role in the design of our clinical trial."

"Autosomal recessive Stargardt disease is a devastating orphan retinal disease for which there is currently no treatment," stated Benjamin Yerxa, PhD, Chief Executive Officer of Foundation Fighting Blindness. "We are excited that Ophthotech has joined the mission to fight against orphan degenerative retinal diseases as both organizations share the common goal of transforming the lives of these patients and their families by developing potential new treatments."

The Company expects to enroll approximately 120 patients in this trial and plans to use an anatomic endpoint, the mean rate of change in the area of ellipsoid zone defect as measured by en face spectral domain optical coherence tomography (SD-OCT), as the primary endpoint, which will be assessed at 18 months. Initial top-line data is expected to be available in 2020.

Ophthotech also announced that it has initiated an open-label Phase 2a clinical trial evaluating Zimura in combination with anti-vascular endothelial growth factor (VEGF) therapy for idiopathic polypoidal choroidal vasculopathy (PCV), an age-related retinal disease. Approximately 20 patients will be enrolled and treated for a duration of 9 months. Initial top-line data is expected to be available during the second half of 2019.

Zimura in Geographic Atrophy, a Severe Form of Dry AMD

An ongoing randomized, double-masked, sham-controlled Phase 2b clinical trial is designed to assess the safety and efficacy of Zimura monotherapy in patients with geographic atrophy (GA) secondary to dry AMD. The Company plans to enroll approximately 200 patients in this trial. The primary efficacy endpoint is the mean rate of change in GA over 12 months. Patients will be treated and monitored for 18 months. A range of Zimura dosing regimens will be assessed. Initial, top-line data is expected to be available during the second half of 2019.

Zimura in Wet AMD

An ongoing dose-ranging, open-label Phase 2a clinical trial of Zimura in combination with Lucentis® will enroll approximately 60 patients with wet AMD who have not been previously treated with any anti-VEGF agents, who will be treated for a duration of 6 months. Based on the anticipated enrollment rate, the Company expects initial top-line data from this trial to be available by the end of 2018.

About Zimura

Zimura is designed to target and inhibit the complement protein C5. Zimura binds to and inhibits C5 from being cleaved into C5a and C5b, potentially preventing the formation of inflammasomes and the accumulation of membrane attack complex (MAC), preventing cell death. Further, when used in combination with anti-VEGF therapy, Zimura may counteract the anti-VEGF induced complement upregulation, thereby providing the rationale as a potential combination therapy for patients with wet AMD and PCV.

About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics for age-related and orphan diseases of the eye. For more information, please visit www.ophtotech.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 timing, progress and results of clinical trials and other development activities, the potential clinical utility of its product candidates, and the potential for its business development strategy. 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, need for additional financing and negotiation and consummation of in-license and/or acquisition transactions 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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Ophthotech Corporation

Investors

Kathy Galante, 212-845-8231

Vice President, Investor Relations and Corporate Communications

kathy.galante@ophthotech.com

or

Media

Alex Van Rees, 973-442-1555 ext. 111

SmithSolve LLC on behalf of Ophthotech Corporation

alex.vanrees@smithsolve.com