



August 5, 2014

## **Ophthotech Initiates First of Several Expansion Studies to Further Evaluate Fovista® (Anti-PDGF) Therapy in Patients with Wet Age-Related Macular Degeneration**

*- First Patient Dosed in an Open-Label Study in Ophthotech's Anti-Fibrosis Program -*

*- Fovista® Combination Therapy Phase 2b Subgroup Analysis for Reduction of Sub-retinal Fibrosis to be Presented at the 2014 Annual Meeting of the American Academy of Ophthalmology -*

NEW YORK--(BUSINESS WIRE)-- Ophthotech Corporation announced today the initiation of the first of several planned expansion trials, in addition to the ongoing Fovista® combination therapy Phase 3 clinical program. These expansion trials will investigate the potential role of Fovista® combination therapy in reducing sub-retinal fibrosis, addressing sub-optimal treatment response and reducing treatment burden in wet age-related macular degeneration (AMD) patients receiving anti-vascular endothelial growth factor (anti-VEGF) monotherapy. The first expansion trial is a Phase 2a open-label study investigating the potential role of anti-platelet derived growth factor (anti-PDGF) therapy in combination with anti-VEGF therapy in reducing sub-retinal fibrosis in wet AMD patients.

"The initiation of the Fovista® expansion studies continues our commitment to advance scientifically-driven therapeutic options to address an unmet medical need in age-related macular degeneration," stated David R. Guyer, M.D., Chief Executive Officer and Chairman of the Board of Ophthotech. "Today, we are pleased to announce the launch of the first study in our expansion trial program investigating the potential effect of administration of Fovista® in combination with an anti-VEGF agent on sub-retinal fibrosis in wet AMD patients. Sub-retinal fibrosis is associated with poor visual outcome in patients receiving anti-VEGF monotherapy for wet AMD. The inhibition of fibrosis is an urgent and unmet medical need in wet AMD."

"Multiple independently published studies have shown that, over time, sub-retinal fibrosis is commonly associated with vision loss in wet AMD patients receiving anti-VEGF monotherapy," stated Samir Patel, M.D., President of Ophthotech. "Peer-reviewed studies also indicate that PDGF is a significant mediator of retinal and organ fibrosis. Coupled with the findings from the retrospective evaluation from our randomized controlled Phase 2b trial, we believe these third party studies provide a strong rationale to investigate whether Fovista® (1.5mg) combination therapy can reduce sub-retinal fibrosis in patients with poor visual outcome, compared to anti-VEGF monotherapy. We are designing the anti-fibrosis expansion trials to provide further information to support the potential use of Fovista® combination therapy for reduction of long term visual loss in wet AMD patients."

The Company also announced that a subgroup analysis showing a reduction of sub-retinal fibrosis and neovascular growth in patients receiving Fovista® (1.5mg) and Lucentis® in the Company's Phase 2b trial has been accepted as an oral presentation at this year's Annual Meeting of the American Academy of Ophthalmology, one of the major medical meetings for retinal physicians. A featured oral presentation entitled, "**Dual Antagonism of Platelet Derived Growth Factor (Fovista® 1.5 mg) and Vascular Endothelial Growth Factor (Lucentis® 0.5 mg) Results in Reduced Sub-retinal Fibrosis and Neovascular Growth**" is scheduled to be presented on October 21, 2014, at the 2014 American Academy of Ophthalmology meeting being held in Chicago, IL.

### **Fovista® Phase 3 Program**

The Fovista® Phase 3 program consists of three clinical trials to evaluate the safety and efficacy of Fovista® (anti-PDGF) therapy, which Ophthotech is developing for use in combination with anti-VEGF drugs for the treatment of wet age-related macular degeneration. The Company expects to enroll up to 1,866 patients in the three trials in more than 225 centers worldwide and to have initial, topline data from the Fovista® Phase 3 clinical program available in 2016.

### **About Wet AMD**

Age-related macular degeneration is a disease characterized by progressive degenerative abnormalities in the macula of the eye, a small area in the central portion of the retina. Age-related macular degeneration is classified into one of two general subgroups: the "dry" (non-neovascular) form of the disease; and the "wet" (exudative or neovascular) form of the disease. The "dry" form of AMD is characterized by a slow degeneration of the macula resulting in atrophy of the central retina, with gradual vision loss over a period of years. By contrast, "wet" AMD typically causes sudden, often substantial, loss of central vision and is

responsible for most cases of severe loss of visual acuity in this disease. Age-related macular degeneration is characteristically a disease of individuals aged 50 years or older, and is the leading cause of blindness in developed countries around the world.

## **About Ophthotech Corporation**

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the eye, with a focus on developing innovative therapies for age-related macular degeneration (AMD). Ophthotech's most advanced product candidate, Fovista® anti-PDGF therapy, is in Phase 3 clinical trials for use in combination with anti-VEGF drugs that represent the current standard of care for the treatment of wet AMD. Ophthotech's second product candidate Zimura™, an inhibitor of complement factor C5, is being developed for the treatment of dry and wet forms of AMD. 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 conduct of the Fovista Phase 3 clinical program, including obtaining initial, top-line data from the Fovista Phase 3 clinical program, the potential of Fovista as a wet AMD combination therapy, the conduct of the first Fovista expansion trial, the initiation of additional clinical trials for Fovista and obtaining data from these trials. 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 and expectations for regulatory approvals or other actions and other important factors discussed in the "Risk Factors" section contained in the quarterly and annual reports that Ophthotech files with the SEC, including Ophthotech's quarterly report on Form 10-Q for the quarter ended March 31, 2014. 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.*

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