



**For Immediate Release**

**Ophthotech's Novel Anti-PDGF Combination Agent Fovista™  
Demonstrated Superior Efficacy over Lucentis® Monotherapy  
in Large Controlled Wet AMD Trial**

***Anti-PDGF (1.5 mg) Combination Therapy Resulted in Additional  
62% Increase in Visual Outcome Compared to Lucentis Monotherapy***

**Princeton, NJ – June 13, 2012** – Ophthotech Corporation today announced results from the first clinical trial to show statistically significant superior efficacy over Lucentis® (ranibizumab) monotherapy for the treatment of neovascular age-related macular degeneration (wet AMD).

In a prospective, randomized, controlled Phase 2b clinical trial of 449 patients with wet AMD, Ophthotech's Fovista™ anti-PDGF therapy (1.5 mg), administered in combination with Lucentis anti-VEGF therapy, met the pre-specified primary efficacy endpoint of mean vision gain. Patients receiving the combination of Fovista (1.5 mg) and Lucentis gained a mean of 10.6 letters of vision on the ETDRS standardized chart at 24 weeks, compared to 6.5 letters for patients receiving Lucentis monotherapy (p=0.019), representing a 62% additional benefit. No significant safety issues were observed for either treatment group in the trial.

Enhanced visual outcomes of Fovista anti-PDGF (1.5 mg) combination therapy as compared to Lucentis monotherapy were demonstrated at every monthly timepoint. In addition, the relative magnitude of visual benefit continued to increase over time. The visual benefit of anti-PDGF (1.5 mg) combination therapy compared to Lucentis monotherapy was greater at the 6-month timepoint than at the 3-month timepoint. The increasing divergence of the efficacy curves suggests the benefit of chronic anti-PDGF combination therapy. A classic dose-response curve was observed.

"This is a truly remarkable finding for patients with wet AMD. To achieve a 62% relative visual benefit over anti-VEGF monotherapy is extraordinary," commented retina specialist Carmen A. Puliafito, M.D., Dean of the Keck School of Medicine at the University of Southern California. "The very compelling and robust results of this well-executed study validate PDGF as an important target for wet AMD and set the stage for a new era of combination therapy via co-formulation or fixed-combination delivery. I look forward to the rapid development of this important drug for our patients."

The robust benefit of Fovista anti-PDGF (1.5 mg) combination therapy over Lucentis monotherapy was consistent across all subgroups including those analyzing baseline vision, lesion size and the proportion of patients gaining 1, 2, 3, 4 and 5 lines of vision (ETDRS standardized chart). An average absolute benefit of 7.4% over Lucentis monotherapy was present across all ETDRS lines of vision gain. In addition, a relative benefit of 25% over Lucentis monotherapy was attained in patients who gained 3 or more lines of vision, with 69% and 178% relative benefit in patients gaining 4 or more and 5 or more lines of vision, respectively.

Donald J. D'Amico, M.D., Professor and Chairman of Ophthalmology at the Weill Cornell Medical College, added, "This breakthrough study is a major step forward in our treatment of patients with wet AMD and represents a clear paradigm shift. This convincing study shows clinically significant improvements in visual outcomes in all patient subgroups over six months. In addition to delivering the promise of enhanced visual gain, I am delighted with the potential of pairing this anti-PDGF entity with any of the increasing number of anti-VEGF agents in the marketplace."

"We are very encouraged by the strong and consistent enhanced efficacy demonstrated in this large trial," stated Samir Patel, M.D., Co-Founder, President and Chief Executive Officer of Ophthotech. "Based on these results, Ophthotech plans to expedite the preparation of a Phase 3 registration program with the goal of bringing Fovista anti-PDGF therapy to patients with wet AMD as soon as possible."

### **About the Phase 2b Trial of Fovista**

The prospective, randomized, controlled Phase 2b trial evaluated the efficacy and safety of Fovista given in combination with Lucentis, compared with Lucentis monotherapy, for the treatment of patients with wet AMD. In this fully masked study, 449 patients were randomized to receive one of the following treatment regimens administered every four weeks for 24 weeks: Fovista 0.3 mg in combination with Lucentis 0.5 mg; Fovista 1.5 mg in combination with Lucentis 0.5 mg; or sham in combination with Lucentis 0.5 mg.

The primary efficacy endpoint in the study was the mean change in visual acuity from baseline at the week 24 visit. As pre-specified in the analysis plan, the Hochberg procedure was employed to account for multiple dose comparisons. Further results will be presented at future medical congresses.

### **About Fovista Anti-PDGF Therapy**

Fovista, formerly known as E10030, is an aptamer directed against platelet-derived growth factor subunit B (PDGF-B), which regulates neovascular pericytes (cells associated with the walls of newly formed small blood vessels). Growth of new blood vessels (neovascularization) is a hallmark of wet AMD. Pharmacology studies indicate that Fovista binds to PDGF-B with high specificity and affinity and inhibits the functions of PDGF-B both *in vitro* and *in vivo*. In preclinical studies involving models of ocular neovascularization, concurrent inhibition of PDGF-B and vascular endothelial growth factor A (VEGF-A) signaling was superior to inhibition of the VEGF-A pathway alone, and demonstrated the potential to induce neovascular regression.

### **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**

Ophthotech Corporation is a privately held biopharmaceutical company based in Princeton, NJ focused on developing and commercializing therapies for dry and wet AMD. Ophthotech is developing a pipeline of compounds with strong scientific foundations for the treatment of AMD, with the goal of bringing them to market in an accelerated manner. Ophthotech's venture investors include SV Life Sciences Advisers, Novo Ventures, HBM Partners and Clarus Ventures. For more information, please visit [www.ophthotech.com](http://www.ophthotech.com).

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