



**FOR IMMEDIATE RELEASE:**

## **Ophthotech Enrolls Its First Patient in a Randomized, Controlled Phase II Study of E10030 in Macular Degeneration**

*Anti-PDGF Aptamer E10030 to be Dosed in Combination with Lucentis®*

**Princeton, NJ – April 26, 2010** – Ophthotech Corp. (“Ophthotech”), a privately held biopharmaceutical company focused on developing ophthalmic therapies for back-of-the-eye diseases, today announced that the first patient has been enrolled in its Phase II randomized, controlled clinical trial of E10030, an anti-PDGF aptamer, in combination with Lucentis® for the treatment of wet age-related macular degeneration (AMD).

The Phase II trial will assess the efficacy and safety of the combination treatment regimen with approximately 444 patients enrolled at sites in the US, Europe and Latin America. In a Phase I clinical study, 59 percent of patients treated with E10030 and Lucentis, an anti-VEGF agent, gained significant vision (3-line gain or better) 12 weeks after the start of therapy. Notably, there was a mean decrease of 86% in the area of choroidal neovascularization (CNV) at 12 weeks. In comparison, current standard of care utilizing monotherapy anti-VEGF treatment does not induce significant neovascular regression and results in 3-line or better visual gain in approximately one third of patients.

"With current standard of care, the majority of wet AMD patients do not achieve significant visual gain nor neovascular regression," said Samir Patel, MD, president and CEO of Ophthotech. "Combination therapy with E10030 demonstrates a significant advancement in the treatment of wet AMD. The magnitude of neovascular regression noted in this Phase 1 study appears to be associated with a significant visual gain in the majority of these patients after only 12 weeks, a result not seen with anti-VEGF treatment alone."

Wet AMD is characterized by the abnormal growth of blood vessels (neovascularization) beneath the retina, which leak blood and fluid and can cause permanent damage to cells in the center of the retina (the macula). This form of AMD is the most severe form of the disease, and often leads to permanent vision loss.

E10030 is an aptamer targeting PDGF-B, a key molecule involved in the recruitment and maturation of pericytes. Pericytes in neovascular tissue have been shown to be protective and play a major role in anti-VEGF treatment resistance. E10030 strips the pericytes from the neovascular tissue rendering it highly sensitive to an anti-VEGF attack.

### **About anti-PDGF E10030**

E10030 is an aptamer-based compound directed against PDGF-B. Pharmacology studies indicate that E10030 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 VEGF-A signaling was superior to inhibition of the VEGF-A pathway alone and demonstrated the potential to regress neovascularization.

E10030 is one of three compounds that Ophthotech is developing to treat wet and dry AMD. Additional molecular entities include an aptamer targeting the complement factor C5 and volociximab, an anti-angiogenic monoclonal antibody targeting the  $\alpha 5\beta 1$  integrin, both currently in Phase I studies.

### **About AMD**

Age-related macular degeneration (AMD) is the leading cause of blindness for people over the age of 50 in the United States and Europe. The role of abnormal neovascularization, or angiogenesis, in the pathogenesis of neovascular AMD has been well established. There are two forms of the disease, namely "dry" and "wet" AMD. The "wet" form is characterized by the growth of new blood vessels into the central region of the retina. These new vessels cause severe visual loss due to retinal damage caused by subsequent leakage and scar formation. Anti-VEGF therapies and photodynamic therapies have been approved for "wet" AMD. "Dry" AMD accounts for up to 90 percent of all cases of AMD. There is no approved therapy for "dry" AMD, which afflicts 8 million patients in the United States and an additional 8 million in Europe. Visual loss in "dry" AMD is typically not as severe as "wet" AMD, however, over time, "dry" AMD can progress to the wet form of the disease.

### **About Ophthotech**

Ophthotech Corp. is a privately held biopharmaceutical company based in Princeton, NJ and New York, NY focused on developing and commercializing therapies for back-of-the-eye diseases. Ophthotech plans to develop a pipeline of compounds with strong scientific foundations for the treatment of AMD and bring them to market in an accelerated manner. For more information, please visit <http://www.ophthotech.com>.