FOVISTA™ (Anti-PDGF) Therapy Fact Sheet

OPHTHOTECH

ABOUT FOVISTA

- Fovista™ is an anti-platelet-derived growth factor (anti-PDGF) agent
- Ophthotech is investigating Fovista™ administered in combination with anti-vascular endothelial growth factor (anti-VEGF) therapy for the treatment of neovascular age-related macular degeneration (wet AMD)
- Fovista™ (1.5 mg) combination therapy showed superior efficacy with statistical significance over anti-VEGF monotherapy (standard of care) for wet AMD in a large (449 patients) superiority Phase 2b trial (see below)

PHASE 3 CLINICAL TRIALS

- Ophthotech has begun treating patients in two of three Phase 3 clinical trials for Fovista™
- Ophthotech plans to enroll 1,866 patients in its Phase 3 clinical trials
- Phase 3 clinical trials build upon and incorporate Phase 2b clinical trial design
- Two of the three Phase 3 clinical trials will evaluate the efficacy and safety of Fovista™ administered in combination with Lucentis®; a third clinical trial will evaluate Fovista™ administered in combination with each of Avastin® or Eylea®
- The primary endpoint, mean change in visual acuity from baseline remains the same as in the Phase 2b clinical trial, but will assess mean change in visual acuity from baseline at 12 months, instead of at 24 weeks as in the Phase 2b

PHASE 2B STUDY DESIGN

- Prospective, randomized, controlled Phase 2b trial in 449 patients with wet AMD
- Evaluated the efficacy and safety of Fovista™ (anti-PDGF) and Lucentis® (anti-VEGF) combination therapy compared to Lucentis® monotherapy
- 3 arm trial: Two doses of Fovista™ (0.3 mg and 1.5 mg) each in combination with Lucentis® were studied compared to the Lucentis® monotherapy arm

PHASE 2B PRIMARY RESULTS

- Primary Endpoint: The pre-specified primary endpoint of superiority (mean change in visual acuity at 24 weeks) was met with statistical significance in patients receiving the combination of Fovista™ (1.5 mg) and Lucentis®
- A 62% comparative benefit from baseline over Lucentis® monotherapy was attained in the Fovista™ (1.5mg) combination arm patients at the week 24 timepoint (primary endpoint). Patients in the Fovista™ (1.5 mg) and Lucentis® combination arm gained a mean of 10.6 ETDRS letters at 24 weeks, compared to 6.5 ETDRS letters for patients receiving Lucentis® monotherapy (p=0.019)
- Safety: Favorable safety profile, no imbalances were observed in any of the treatment groups with respect to ocular and systemic adverse events or persistent intraocular pressure elevation
- A classic dose-response curve favoring the Fovista™/Lucentis® combination arms was observed for the primary endpoint at all monthly visits (mean change in visual acuity)
- The relative magnitude of visual benefit continued to increase over time, favoring the patients receiving the Fovista™ (1.5 mg) combination therapy
- All significant heterogeneous treatment endpoints of visual benefit favored the patients receiving the Fovista™ (1.5 mg) combination therapy (Endpoints: >3, 4 and 5-ETDRS line gain; Final vision outcome of 20/40 and 20/25 or better)
- Patients receiving the Fovista™ (1.5 mg) combination therapy experienced less visual loss than Lucentis® monotherapy patients. All significant heterogeneous treatment endpoints of visual LOSS, at week 24, favored Fovista™ (1.5 mg) combination therapy (Endpoints: >1 or 2-ETDRS line loss; Final vision outcome of 20/125 and 20/200 or worse)
- Disease modification (decrease in neovascular complex size) was noted by independent masked readers, and in a restrospective subgroup analysis, Ophthotech observed that patients that received Fovista™ (1.5mg) combination therapy , exhibited a greater mean change in area of neovascularization in two subgroups for which neovascularization data were available both at trial baseline and at 24 weeks