

Dermatologic and Ophthalmic Drugs Advisory Committee

27 August 2004

Questions for the Advisory Committee Meeting

- Has sufficient data been submitted to evaluate the efficacy and safety profile of pegaptanib sodium? If not, what additional data are needed?
- Based on the Inclusion/Exclusion Criteria, are there patients excluded from the studies that you believe need to be studied?
- Are additional analyses of the current data needed to understand the efficacy or safety of pegaptanib sodium for the treatment of age-related macular degeneration?
- Visual acuity measurements were conducted using the ETDRS scale placed at 2 meters from the patient. The validity of the ETDRS scale was established based on readings at 4 meters. Are the visual acuity findings sufficiently robust to overcome the potential bias introduced by visual acuity measurements at 2 meters?
- Has the concomitant use of PDT therapy with pegaptanib been explored sufficiently? Are there concerns with using this product concomitantly with PDT therapy?
- Do the route and/or frequency of administration of the drug raise any concerns that are not addressed by the studies?
- Are there adverse experiences that are of particular concern for this product?
- Endophthalmitis (approximately 2%) was observed in these studies. What is the optimal follow-up needed to minimize the impact of potential endophthalmitis cases?
- Vascular Endothelial Growth Factor (VEGF) has been shown to be an important component in the development of collateral vessels in ischemic heart disease. Inhibition of VEGF in the systemic circulation could present a theoretical increased risk of symptomatic cardiovascular disease in the target population of elderly patients with AMD. Has the adverse event profile of the two randomized phase 3 trials raised any concern over the possible systemic effects of this therapy? Is there additional monitoring that should be in place for patients on pegaptanib sodium therapy?
- Do the benefits of using pegaptanib sodium outweigh the risks in the treatment of age-related macular degeneration?