

Efficacy and safety of intravitreal pegcetacoplan in geographic atrophy: Results from the phase 3 DERBY and OAKS trials

Purpose: Currently, no approved therapies exist to slow the progression of geographic atrophy (GA). Intravitreal pegcetacoplan, a pegylated complement C3 inhibitor peptide, demonstrated significant reductions in the growth of GA lesions compared with sham treatment in a phase 2 trial. DERBY and OAKS are two 24-month, phase 3, randomised, double-masked, sham-controlled clinical trials comparing the efficacy and safety of monthly or every-other-month intravitreal pegcetacoplan to sham in patients with GA secondary to age-related macular degeneration

Methods: Enrolled patients are ≥ 60 years old, have best-corrected visual acuity ≥ 24 letters, and GA area between 2.5 and 17.5 mm² or one focal lesion ≥ 1.25 mm² if multifocal GA at baseline. The primary endpoint for both studies is change in GA lesion size via fundus autofluorescence from baseline to month 12; secondary endpoints include change from baseline in visual function. Safety measures include incidences of ocular and systemic adverse events.

Results: DERBY and OAKS enrolled N = 621 and N = 638 patients, respectively. Baseline characteristics were well-matched across the groups. Enrolment was completed in June 2020 (DERBY) and July 2020 (OAKS). Twelve-month efficacy and safety data will be presented.

Conclusions: Pegcetacoplan is the only targeted C3 inhibitor being evaluated in phase 3 trials to control lesion growth in GA.