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DESIGN OF A GLOBAL PHASE 2 RANDOMIZED, PLACEBO-CONTROLLED TRIAL OF THE ORAL FACTOR D INHIBITOR DANICOPAN IN GEOGRAPHIC ATROPHY

Poster

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Purpose:

Geographic atrophy (GA) secondary to age-related macular degeneration (AMD) is a progressive degenerative disease leading to vision loss that lacks approved therapies. A randomized, placebo-controlled trial (RCT) is evaluating the efficacy and safety of the oral complement factor D inhibitor danicopan in patients with GA.

Methods:

A global phase 2 RCT with 6-week screening and 104-week double-masked treatment is enrolling 332 patients ≥60 years with non-center involving GA in ≥1 eye (lesions: 0.5-17.76 mm2) and without neovascular AMD in the study eye (EudraCT: 2021-001198-22). Randomization is 1:1:1:1 to danicopan 100 or 200 mg BID, 400 mg QD, or placebo. The primary endpoint is change from baseline in square root of total GA lesion area at week 52. The trial includes a range of secondary outcome measures, including best-corrected and low-luminance visual acuity scores, and incidence rates of treatment-related adverse events.

Results:

The RCT is currently enrolling participants in multiple countries.

Conclusions:

This is the first global phase 2 RCT to evaluate the efficacy and safety of an oral factor D inhibitor in patients with GA.