

## **A Phase 2 Study of Encapsulated CNTF Secreting Cell Implant (NT-501) in Patients with Geographic Atrophy Associated with Dry AMD**

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### **Purpose**

Currently, no effective treatment is available for vision loss associated with geographic atrophy. NT-501 protects photoreceptors in a number of animal models. The purpose of the present study is to evaluate the safety and efficacy of NT-501 in a multi-center, double-masked, controlled, dose-ranging and randomized phase 2 trial for geographic atrophy associated with dry AMD.

### **Method**

The CNTF study consists of 48 participants that were randomized in a 2:1:1 ratio to receive an intravitreal high or low CNTF output implant, or to sham surgery, respectively. BCVA was measured by an Electronic Visual Acuity Tester (EVA) using the Early Treatment Diabetic Retinopathy Study (ETDRS) protocol, retinal thickness was measured by time domain optical coherence tomography (OCT) and lesion size was measured by fundus photography, each at 4, 6, and 12 months following implant placement. CNTF release from explanted implants was measured by ELISA.

### **Results**

CNTF treatment resulted in a dose-dependent increase in retinal thickness as early as 4 months post-implant and this increase was maintained through 6 and 12 months ( $p < 0.001$ ). This anatomical change was associated with visual function stabilization, as measured by 15-letter loss, in the high dose-treated group compared to sham and low dose groups at 12 months. Among eyes with starting BCVA 20/63 the mean BCVA in the high dose group was 10.5 letters greater than the low dose/sham group ( $p < 0.03$ ). Both the NT-501 implant and the implant procedure were well tolerated; no serious adverse events associated with the implant or implantation procedure were reported. The explanted devices showed healthy cells and stable CNTF output up to 12 months.

### **Conclusion**

Intraocular CNTF delivered by ECT produced long-term increased retinal thickness and stabilized visual acuity, without serious adverse events, in eyes with geographic atrophy associated with non-neovascular AMD. These data support next stage clinical studies in a larger patient population.