

Positive Results from Dry AMD (Geographic Atrophy) Study

Neurotech Pharmaceuticals, Inc., announced on May 25 that the Company's lead product candidate, NT-501, substantially slowed the loss of vision in a Phase 2 clinical trial in subjects with dry age-related macular degeneration (AMD) involving geographic atrophy (GA). GA is a condition that destroys sharp central vision, often resulting in serious vision loss to one or both eyes. There are currently no approved treatments for dry AMD. In the study, the high dose of NT-501 stabilized best corrected visual acuity (BCVA) at 12-months, with 96.3% ($p=0.078$) of treated-patients losing fewer than three lines of vision, or 15 letters, versus 75% of the patients in the sham-treatment group. NT-501 is an intraocular implant that consists of human cells that have been genetically modified to secrete ciliary neurotrophic factor (CNTF). CNTF is delivered directly to the back of the eye in a controlled, continuous basis by means of the Encapsulated Cell Technology (ECT) platform, thereby bypassing the blood-retinal barrier and overcoming a major obstacle in the treatment of retinal disease.

The Retina Foundation of the Southwest was a lead center in the multi-centered, randomized, double-masked, sham-controlled study of 51 subjects with GA. Patients received either a high or low dose NT-501 implant or a sham treatment in one eye only and were assessed for changes in best-corrected visual acuity, which was measured by an Electronic Visual Acuity Tester (EVA). Patients were also evaluated for an increase in BCVA. However, no increase was observed, likely due to existing photoreceptor damage. There were no NT-501 associated serious adverse events reported and both NT-501 and the surgical procedure were well-tolerated.

"We are extremely encouraged that we may be slowing visual loss for patients with this advanced stage of dry AMD. Finding effective intervention is particularly important given the prevalence of this form of AMD and its negative impact on quality of life" stated Dr. David G. Birch, a study investigator and Director of the Rose-Silverthorne Laboratory at the RFSW.

The strong trend in visual acuity stabilization at 12 months was preceded by a dose-dependent, statistically significant ($p<0.001$ and $p=0.013$ for high and low dose, respectively) increase in retinal thickness as measured by optical coherence tomography (OCT) that was observed as early as 4 months post-implantation. The observed structural change is consistent with preclinical studies of NT-501 in which CNTF was shown to increase the thickness of the retina and the outer nuclear layer of photoreceptors responsible for vision. This increase in retinal thickness may be responsible for photoreceptor rescue and protection as observed in numerous animal models of retinal degeneration.

"Based on the increase in retinal thickness observed in this study it appears that CNTF may be exhibiting a biological effect on retinal photoreceptors as has been observed previously in animal studies," said Dr. Paul Sieving, Director of the National Eye Institute and Principal Investigator of Neurotech's Phase 1 study of NT-501 in retinitis pigmentosa.

“We believe the anatomical changes observed in patients treated with NT-501 have led to the emergence of a clinically meaningful visual acuity benefit for patients with geographic atrophy,” commented Ted Danse, President and Chief Executive Officer of Neurotech. “NT-501 may provide a much needed treatment option for these patients and we intend to discuss these data and a pivotal trial design with the FDA.”

“We are very pleased that the outcome of this trial has shown such promise for patients with dry AMD involving geographic atrophy and are proud of our long-term support for this unique, breakthrough technology,” stated Stephen Rose, PhD, Chief Research Officer, Foundation Fighting Blindness.

Five devices from this trial have been explanted 12 months following implantation and all have been found to have uniformly healthy, viable cells that continue to produce therapeutic levels of CNTF. This is consistent with data from multiple trials of NT-501 in which, to date, 23 devices have been explanted between 12 and 18 months following implantation and all devices have contained healthy, viable CNTF-producing cells. “We also believe the positive results of this study and long-term cell viability validate our ECT platform and support a breakthrough opportunity to advance long-term, well-tolerated treatments for patients facing chronic sight-stealing retinal diseases. As such, we are developing our second product utilizing the ECT platform to address a well-validated target, anti-VEGF therapy for wet AMD, that has the potential to provide a one-time administration for a 12 to 18 month period versus the current wet AMD treatment regimen that requires monthly injections with routine patient monitoring,” concluded Danse.