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Neurotech Pharmaceuticals, Inc. and Lowy Medical Research Institute Announce Publication of NT-501 Phase 2 Results

January 16, 2019, Cumberland, RI and La Jolla, CA. The results of the NT-501 Phase 2 study sponsored by **Neurotech Pharmaceuticals, Inc. (Neurotech)** in collaboration with the **Lowy Medical Research Institute (LMRI)**, have been published in the journal of the American Academy of Ophthalmology, *Ophthalmology*[1]. The study, a Phase 2 multi-center randomized clinical trial, tested the effect of encapsulated cell-based delivery of ciliary neurotrophic factor (CNTF) on retinal degeneration in patients with macular telangiectasia type 2 (MacTel). The paper was authored by a group of clinicians and scientists who collaborated on the project through the Macular Telangiectasia Type 2-Phase 2 CNTF Research Group.

MacTel type 2 is a rare macular degenerative disease typically diagnosed in middle age. It is primarily a neurodegenerative disease, but also affects Müller glia and blood vessels. Patients rarely experience total vision loss, but the disease nonetheless has a significant impact, through visual loss, on a patient's quality of life. LMRI supports MacTel type 2 clinical and laboratory research and collaborated with Neurotech to test NT-501 in MacTel type 2 patients.

The NT-501, or Renexus® device, is manufactured by Neurotech. It is a novel cell-based drug delivery system. Cells encapsulated in a semipermeable hollow fiber membrane release ciliary neurotrophic factor (CNTF); CNTF has been shown to reduce photoreceptor cell loss in animal models of retinal degeneration. The implanted Renexus® device results in sustained delivery of CNTF localized to the retina, the light-sensing tissue in the back of the eye.

The *Ophthalmology* report describes the results of a single-masked, randomized clinical trial that tested the effects of CNTF delivered by NT-501 in participants with MacTel type 2. Participants were randomized 1:1 to surgical implantation of the NT-501 device, or sham procedure. Measures of photoreceptor health and function were assessed 24 months from patient enrollment. The study showed that NT-501 treatment slowed the progression of retinal degeneration compared with participants who were given the sham treatment. In addition, NT-501 provided functional benefit; reading speed was stabilized in patients receiving the implant.

Patients from the Phase 1 and 2 studies continue to be followed to evaluate the long-term effect of CNTF on retinal degeneration.

Dr. Martin Friedlander, President of Lowy Medical Research Institute, noted that "the mission of LMRI is to understand MacTel and bring therapies to market for this disease. The Phase 2 NT-501 clinical trial and extension study results are very encouraging, and bring us closer to the important goal of providing efficacious treatment options for people diagnosed with MacTel, a disease for which we currently have no treatments."

Renexus® is also being studied by Neurotech to test the neuroprotective effects of CNTF in treating glaucoma. Renexus® is currently being tested in a randomized, sham controlled, masked Phase 2 glaucoma study in 54 patients. The glaucoma study is independent of the MacTel research project.

"There is currently no effective therapy available to MacTel type 2 patients", said Richard Small, CEO of Neurotech. "We are encouraged by the neuroprotective effect of NT-501 observed in the MacTel phase 2 trial and associated extension study. A Phase 3 study to determine the safety and efficacy of Renexus ® in MacTel type 2 is currently enrolling patients in the United States, Australia, and Europe." (Clinicaltrials.gov #NCT03319849/NCT03316300)

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About Macular Telangiectasia

Macular telangiectasia (MacTel), or idiopathic juxtafoveal macular telangiectasia, is a rare neurodegenerative disease with characteristic alterations of the retinal vasculature and localized retinal degeneration. There are three classifications of MacTel, describing distinct clinical entities. Type 2 is the most common classification, afflicting approximately 1 in 22,000 individuals, with most patients diagnosed in their 40s and 50s. MacTel type 2 typically affects both eyes, and results in deterioration of central vision over a period of 10 to 20 years.

About Encapsulated Cell Therapy

Encapsulated Cell Therapy (ECT) is an investigational, first in class, versatile delivery system that promotes continuous production of therapeutic proteins to the eye with the potential to treat a broad array of ocular diseases. It utilizes a proprietary, well-characterized retinal pigment epithelial cell line that has been genetically engineered to produce therapeutically active biologics. The cells are encapsulated in a semi-permeable membrane that allows for selective passage of therapeutic proteins. The ECT platform is inserted during a single outpatient surgical procedure through a small scleral incision, and can also be removed through the same incision, if desired. ECT has the potential to address the current limitations of intraocular drug delivery by reducing treatment burden with one surgical procedure that can deliver drug for at least 2 years.

About Lowy Medical Research Institute

The Lowy Medical Research Institute (LMRI) is a private, non-profit biomedical research organization dedicated to the study of MacTel type 2. LMRI was established in 2005 to act as the parent organization and funding agency for the MacTel Project, which was initiated the same year. The MacTel Project's Natural History Observation Study enrolled more than 400 individuals from around the world and has led to new insights into the disease. LMRI also supports a patient registry, in which more than 1,500 participants have enrolled, providing valuable clinical information about disease progression and opportunities for patients and their family members to participate in laboratory research and clinical trials. To learn more, visit www.lmri.net.

About Neurotech Pharmaceuticals, Inc.

Neurotech Pharmaceuticals, Inc. is a private biotechnology company focused on developing transformative therapies for chronic eye diseases. The core technology platform, ECT, enables continuous production of therapeutic proteins to the eye. Neurotech is currently studying in the clinic ECT candidates to treat macular telangiectasia and glaucoma. To learn more, visit www.neurotechusa.com.

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[1]The Macular Telangiectasia Type 2-Phase 2 CNTF Research Group, Chew EY, Clemons TE, Jaffe GJ, Johnson CA, Farsiu S, Lad EM, Guymer R, Rosenfeld P, Hubschman J-P, Constable I, Wiley H, Singerman LJ, Gillies M, Comer G, Blodi B, Eliott D, Yan J, Bird A, Friedlander M, Effect of Ciliary Neurotrophic Factor on Retinal Neurodegeneration in Patients with Macular Telangiectasia Type 2: A Randomized Clinical Trial, *Ophthalmology* (2018), doi: https://doi.org/10.1016/j.ophtha.2018.09.041.