Neurotech Announces Positive Phase 2 Results in NT-501 (CNTF) for Macular Telangiectasia

NT-501 Slows Progression of Disease over 24 months

June 20, 2017, Cumberland, RI – Neurotech Pharmaceuticals, Inc., in collaboration with the Lowy Medical Research Institute (LMRI), today announced 24-month results demonstrating that NT-501 delivering Ciliary Neurotrophic Factor (CNTF) has a beneficial effect in patients with Macular Telangiectasia type 2 (MacTel). The multicenter, randomized clinical trial demonstrated a statistically significant reduction in the progressive loss of photoreceptors in treated versus untreated eyes. NT-501 utilizes the Company's proprietary Encapsulated Cell Therapy (ECT) platform that can be customized to deliver specific therapeutic molecules to the back of the eye for retinal disease.

The Phase 2 study enrolled 67 patients (99 eyes) at eight sites in the United States and three in Australia. Eligible eyes were randomized to receive either the NT-501 implant containing CNTF or a sham procedure. The primary endpoint, change in the ellipsoid zone from baseline to month 24, was measured by Spectral Domain Optical Coherence Tomography (SD-OCT). An increase in the area of the ellipsoid zone is a measure of disease progression in patients with MacTel and is correlated with loss of photoreceptors leading to visual loss.

At 24 months, there was significantly less photoreceptor loss in eyes treated with NT-501 versus sham. The area of ellipsoid zone break increased by 0.213 mm^2 in sham eyes compared to 0.148 mm^2 in treated eyes. The difference in the increase of the MacTel lesion (0.065 mm^2) was statistically significant (p=0.030). The proportion of study eyes with a 35% or more increase from baseline in the ellipsoid zone was significantly reduced in the treated group (p = 0.045) and macular thickness was significantly increased in the treated population when compared to the control group (p=0.007). Secondary clinical outcomes showed reading speed being maintained in the study eyes while deteriorating in the sham eyes (p=0.016).

Martin Friedlander, M.D., Ph.D., President of the LMRI commented, "The results of the Phase 2 study are very encouraging and support plans to progress NT-501 to a Phase 3 clinical trial. NT-501 appears to slow the rate of progression of the disease and if additional studies replicate the Phase 2 data, this therapy has the potential to become the first treatment available for MacTel".

NT-501 was generally well tolerated, consistent with previous studies of NT-501 in retinitis pigmentosa and dry AMD. No participants had the implant removed during the course of the study. The majority of adverse events were related to the surgical procedure. In all cases these surgery-related events were resolved within 3 months.

"We are very excited in moving forward with the MacTel clinical program and are planning to initiate the Phase 3 program by the end of this year", said Richard Small, Chief Operating Officer at Neurotech. "In addition, we are looking forward to seeing the results of our NT-501 clinical program in glaucoma by early next year".

About Macular Telangiectasia and the Lowy Medical Research Institute

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 causes patients to lose central vision over a period of 10 to 20 years.

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 1,000 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 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 allowing for single- and multiple-factor drug combinations and ensuring patient compliance and reducing treatment burden with one surgical procedure that can deliver drug for at least 2 years.

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.