



Neurotech Pharmaceuticals, Inc. Granted Fast Track Designation from the U.S. FDA for the Treatment of Macular Telangiectasia type 2.

Cumberland, RI., February 12, 2019 – Neurotech Pharmaceuticals, Inc. (Neurotech), a clinical-stage biopharmaceutical company focused on the development of transformative therapies for chronic eye diseases, announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the Company’s development candidate, NT-501 or Renexus® for the treatment of macular telangiectasia type 2 (MacTel). Fast track is a designation by the FDA to facilitate the development and expedite the review of drugs to treat serious or life-threatening conditions and fill an unmet medical need.

Renexus® is a novel cell-based drug delivery system. Human-derived cells encapsulated in a semipermeable hollow fiber membrane device release ciliary neurotrophic factor (CNTF) demonstrated 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. MacTel is a rare macular degenerative disease typically diagnosed in middle age. Patients rarely experience total vision loss, but the disease nonetheless has a significant impact, through visual loss, on a patient’s quality of life.

In a multi-center, randomized, controlled Phase 2 clinical study of MacTel, Renexus® was shown to slow the progression of retinal degeneration compared with participants who received the sham treatment. Based on the positive Phase 2 results, two parallel Phase 3 studies were initiated and are currently enrolling patients in the United States, Australia, and Europe to determine the safety and efficacy of Renexus® for the treatment of MacTel ([NCT03319849](#), [NCT03316300](#)). These studies are sponsored by Neurotech in collaboration with the Lowy Medical Research Institute (LMRI).

“Fast Track designation is very helpful to us in supporting our clinical program to develop an effective treatment for MacTel patients. Currently there are no treatment options available for MacTel patients”, said Richard Small, Chief Executive Officer of Neurotech. “Fast Track offers several key advantages, including the opportunity to work closely with the FDA through more frequent meetings and communications, eligibility for a rolling submission of completed sections of the Biologic License Application (BLA) and potential eligibility for Accelerated Approval and Priority Review”.

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 device 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 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 over an extended period. Neurotech is currently studying in the clinic ECT candidates to treat macular telangiectasia and glaucoma. To learn more, visit www.neurotechusa.com.

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 2013 to act as the parent organization and funding agency for the MacTel Project, which was initiated in 2005. The MacTel Project includes a natural history observation study, a registry program, collaborative genetics and laboratory research programs, and an eye donor program. More than 40 centers around the world participate in the MacTel Project and has enrolled more than 400 individuals from around the world, leading 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. LMRI staff work together to execute and coordinate all research funded by LMRI, including clinical trials, clinical research, community service programs and laboratory research. To learn more, visit www.lmri.net.