Neurotech's ENCELTO™ (revakinagene taroretcel-lwey) Approved by the FDA for the Treatment of Macular Telangiectasia Type 2 (MacTel)

March 6, 2025

- ENCELTO is the First and Only FDA-approved Treatment for MacTel

Cumberland, R.I., March 6, 2025 – Neurotech Pharmaceuticals, Inc., a private biotech company focused on developing transformative therapies for chronic eye diseases, announced that the U.S. Food and Drug Administration (FDA), has approved ENCELTO™ (revakinagene taroretcel-lwey) for the treatment of Macular Telangiectasia type 2 (MacTel).

MacTel is a neurodegenerative disease of the retina in adults that causes progressive and irreversible vision loss, significantly impacting patients' quality of life. ENCELTO utilizes an encapsulated cell therapy technology designed to continually deliver therapeutic doses of ciliary neurotrophic factor (CNTF) to the retina to assist in slowing the progression of the disease. ENCELTO is the first and only FDA-approved treatment available for MacTel.

The approval was based on results from two phase 3 trials which demonstrated that after placement, of the implant, ENCELTO significantly slowed the loss of macular photoreceptors in MacTel patients over 24 months.

ENCELTO is expected to be available in the United States for patients starting in June of 2025.

“Today marks an extraordinary milestone for patients, the retina community, and Neurotech,” said Richard Small, Chief Executive Officer. “I would like to express my gratitude to clinical study participants, clinical investigators and their teams, and the entire Neurotech organization who have helped make this a reality.”

“I have seen the impact that MacTel can have on patients and their quality of life,” said Charles C. Wykoff, MD, PhD, Retinal Consultants of Texas, Houston, TX, a clinical investigator. “Now with an FDA-approved treatment, I am confident that ENCELTO will be able to meaningfully slow disease progression for many patients affected by MacTel, allowing them the opportunity to preserve more functional vision over time.”

"This is a historic moment for the MacTel community, as ENCELTO becomes the first-ever FDA-approved treatment for this vision-threatening disease,” said Thomas M. Aaberg Jr, MD, Chief Medical Officer. “For those who have been affected by MacTel and for all who have supported this journey, today we look forward to a future where vision loss from MacTel may be slowed."

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use ENCELTO™ safely and effectively. See full prescribing information for ENCELTO.

ENCELTO (revakinagene taroretcel-lwey) implant, for intravitreal use
Initial U.S. Approval: 2025

----------**INDICATIONS AND USAGE**----------

ENCELTO is an allogeneic encapsulated cell-based gene therapy indicated for the treatment of adults with idiopathic macular telangiectasia type 2 (MacTel).

----------**DOSAGE AND ADMINISTRATION**----------

For intravitreal implantation only.

- ENCELTO is intended for surgical intravitreal implantation under aseptic conditions by a qualified ophthalmologist.
- The recommended dose is one ENCELTO implant per affected eye containing 200,000 to 440,000 allogeneic retinal pigment epithelial cells expressing recombinant human ciliary neurotropic factor (rhCNTF).
- Carefully inspect ENCELTO prior to use and refer to the Instructions for Use when preparing for and performing surgical placement or removal of ENCELTO.

----------**DOSAGE FORMS AND STRENGTHS**----------

One single-dose implant containing 200,000 to 440,000 allogeneic retinal pigment epithelial cells expressing rhCNTF.

----------**CONTRAINDICATIONS**----------

- Ocular or periocular infections.
- Known hypersensitivity to Endothelial Serum Free Media (Endo-SFM).

----------**WARNINGS AND PRECAUTIONS**----------

- ENCELTO implantation has been associated with severe vision loss, infectious endophthalmitis, retinal tears and/or detachment, vitreous hemorrhage, implant extrusion, cataract formation, suture related complications, and delayed dark adaptation. Patients should be instructed to report signs or symptoms that could be associated with these events without delay. Additional surgical and/or medical management may be required.
- Vitreous Hemorrhage: Temporarily discontinue antithrombotic medication prior to ENCELTO insertion surgery to reduce the risk of implantation related vitreous hemorrhage. Vitreous hemorrhages occurring greater than one year from implantation could be a sign of ENCELTO extrusion. The surgical site should be examined closely and the ENCELTO should be surgically repositioned if indicated.

----------**ADVERSE REACTIONS**----------

The most common adverse reactions (incidence ≥2%) were conjunctival hemorrhage, delayed dark adaptation, foreign body sensation, eye pain, suture related complications, miosis, conjunctival hyperemia, eye pruritus, ocular discomfort, vitreous hemorrhage, blurred vision, headache, dry eye, eye irritation, cataract progression or formation, vitreous floaters, severe vision loss, eye discharge, anterior chamber cell, iridocyclitis.

To report SUSPECTED ADVERSE REACTIONS, contact Neurotech at 1-833-963-9275 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

About Macular Telangiectasia Type 2 (MacTel)

Macular Telangiectasia Type 2 (MacTel), or idiopathic macular telangiectasia type 2, is a bilateral, neurodegenerative disease in adults with characteristic localized retinal degeneration, a group of diseases that cause the gradual loss of cells in the retina, resulting in vision loss and secondary alterations of the retinal vasculature, the network of blood vessels that supplies oxygen and nutrients to the retina.[1]

About Encapsulated Cell Therapy (ECT)

Neurotech's ECT platform is a cell-based gene therapy delivery system designed to provide long-term, sustained delivery of therapeutic proteins for the treatment of chronic eye diseases. This versatile platform consists of a small, semi-permeable capsule containing proprietary allogeneic retinal pigment epithelium (RPE) cells genetically engineered to produce specific therapeutic proteins for targeted disease treatment. The capsule is surgically implanted. Once in place, the capsule's semi-permeable exterior membrane allows essential nutrients to enter, while also permitting the therapeutic proteins to exit into the eye where they can travel to the retina located at the back of the eye. The exterior membrane protects the encapsulated RPE cells from the host's immune system, contributing to their survival and functionality over time.

About Neurotech Pharmaceuticals, Inc

Neurotech Pharmaceuticals, Inc. is a private biotech company focused on developing transformative therapies for chronic eye diseases. The core platform technology, Encapsulated Cell Therapy (ECT) is a first in class, drug delivery platform designed to slow the progression of MacTel and other chronic eye diseases. For more information about us, please visit https://www.neurotechpharmaceuticals.com.

1.Charbel Issa P, Gillies MC, Chew EY, Heeren TFC, Bird AC, Peto T, Holz FG, Scholl HPN (2013) Macular Telangiectasia Type 2.

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