Encelto™ (revakinagene taroretcel-lwey) – New orphan drug approval

- On March 6, 2025, Neurotech announced the FDA approval of Encelto (revakinagene taroretcel-lwey), for the **treatment of adults with idiopathic macular telangiectasia type 2 (MacTel).**

- MacTel is a neurodegenerative disease of the retina in adults that causes progressive and irreversible vision loss.

- **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 FDA approved treatment available for MacTel.

- The efficacy of Encelto was established in Study 1, a randomized, sham-controlled study in 120 adults with MacTel. Patients were randomized to receive either Encelto intravitreal implant or sham procedure under standard operative procedures. The primary outcome measure was the rate of change in the area of ellipsoid zone (EZ) loss over 24 months.

    — The rate of change in EZ area loss from baseline over 24 months was 0.075 mm^2 and 0.166 mm^2 with Encelto and sham, respectively (difference -0.091, 95% CI: -0.13, -0.06; $p < 0.0001$).

- The efficacy of Encelto was also established in Study 2, a randomized, sham-controlled study in 119 adults with MacTel. Patients were randomized to receive either Encelto intravitreal implant or sham procedure under standard peri-operative procedures. The primary outcome measure was the rate of change in the area of EZ loss over 24 months.

    — The rate of change in EZ area loss from baseline over 24 months was 0.111 mm^2 and 0.160 mm^2 with Encelto and sham, respectively (difference -0.049, 95% CI: -0.089, -0.008; $p < 0.0186$).

- Encelto is contraindicated in patients with:

    — Active or suspected ocular or periocular infections.
    — Known hypersensitivity to Endothelial Serum Free Media.

- Warnings and precautions for Encelto include severe vision loss; infection endophthalmitis; retinal tear and detachment; vitreous hemorrhage; implant extrusion; cataract formation; suture related complications; and delayed dark adaption.

- The most common adverse reactions (≥ 2%) with Encelto use 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, and iridocyclitis.

- **The recommended dose of Encelto is one implant per affected eye**. Each Encelto implant contains 200,000 to 440,000 allogeneic retinal pigment epithelial cells expressing recombinant human ciliary neurotropic factor (rhCNTF).

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  - Encelto is administered by a single surgical intravitreal procedure performed by a qualified ophthalmologist.

- Neurotech plans to launch Encelto in June 2025. Encelto will be available as a single-dose implant containing 200,000 to 440,000 allogeneic retinal pigment epithelial cells expressing rhCNTF.