Clinical Policy: Revakinagene Taroretcel-lwey (Encelto)

Reference Number: CP.PHAR.697
Effective Date: 03.05.25
Last Review Date: 05.25
Line of Business: Commercial, HIM, Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description

Revakinagene taroretcel-lwey (Encelto™) is an allogeneic encapsulated cell-based gene therapy.

FDA Approved Indication(s)

Encelto is indicated for the treatment of adults with idiopathic macular telangiectasia type 2 (MacTel).

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

All requests reviewed under this policy **require medical director review**.

It is the policy of health plans affiliated with Centene Corporation® that Encelto is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Idiopathic Macular Telangiectasia Type 2 (must meet all)**:**

1. Diagnosis of MacTel confirmed by both of the following (a and b):
   a. Fluorescein angiographic leakage of the retinal vessels;
   b. One of the following (i, ii, iii, iv, or v):
      i. Retinal opacification;
      ii. Crystalline deposits;
      iii. Right angle vessels;
      iv. Inner/outer lamellar cavities;
      v. Hyperpigmentation not involving the foveal center;
2. Prescribed by or in consultation with a retina specialist;
3. Age ≥ 18 years;
4. Ellipsoid zone (EZ) disruption between 0.16 mm^2 and 2.00 mm^2 as measured by optical coherence tomography (OCT);
5. Best corrected visual acuity (BCVA) of 54 letters or better on Early Treatment Diabetic Retinopathy Study (ETDRS) charts (approximately 20/80 Snellen equivalent);
6. Member does not have intraretinal or subretinal neovascularization;
7. Member has not previously received an ocular implant containing Encelto in the affected eye(s);
8. Dose does not exceed 1 ocular implant per eye.

Approval duration: 3 months (one implant per eye per lifetime)

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Idiopathic Macular Telangiectasia Type 2
1. Re-authorization is not permitted for previously treated eyes. If request is for treatment of an eye that has not previously received an ocular implant, members must meet the initial approval criteria.

Approval duration: Not applicable

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

BCVA: best corrected visual acuity
ETDRS: Early Treatment Diabetic Retinopathy Study
EZ: ellipsoid zone
FDA: Food and Drug Administration
MacTel: idiopathic macular telangiectasia type 2
OCT: optical coherence tomography
rhCNTF: recombinant human ciliary neurotrophic factor

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): ocular or periocular infections, known hypersensitivity to Endothelial Serum Free Media (Endo-SFM)
- Boxed warning(s): none reported

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MacTel	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 neurotrophic factor (rhCNTF). Encelto is intended for surgical intravitreal implantation under aseptic conditions by a qualified ophthalmologist.	1 implant/eye

VI. Product Availability

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

VII. References

1. Encelto Prescribing Information. Cumberland, RI: Neurotech Pharmaceuticals, Inc.; March 2025. Available at: https://www.fda.gov/media/185726/download?attachment. Accessed March 6, 2025.
2. Mozayan E, Shah VA, Kim LA, et al. Macular telangiectasia. American Academy of Ophthalmology EyeWiki. Available at: https://eyewiki.aao.org/Macular_Telangiectasia. Last reviewed November 28, 2024. Accessed March 10, 2025.
3. Neurotech Pharmaceuticals. A study to determine the safety and efficacy of NT-501 in macular telangiectasia type 2 – Protocol B. ClinicalTrials.gov. Available at: https://clinicaltrials.gov/study/NCT03319849. Accessed March 10, 2025.

4. Neurotech Pharmaceuticals. A study to determine the safety and efficacy of NT-501 in macular telangiectasia type 2 – Protocol A. ClinicalTrials.gov. Available at: https://clinicaltrials.gov/study/NCT03316300. Accessed March 10, 2025.
5. Chew EY. Phase 3 randomized studies of efficacy and safety of revakinagene taroretcel producing ciliary neurotrophic factor (CNTF) in macular telangiectasia type 2. American Society of Retinal Specialists (ASRS) 41st Annual Meeting; July 28-August 1, 2023; Seattle, USA.
6. Gillies MC. Phase 3 randomized studies of ciliary neurotrophic factor-producing revakinagene taroretcel to treat MacTel2. American Academy of Ophthalmology (AAO) 127th Annual Meeting; November 3-6, 2023; California, USA.
7. Albini TA. Phase 3, multicenter, randomized, sham-controlled studies of the efficacy and safety of revakinagene taroretcel in macular telangiectasia type 2. Retina World Congress 2024; May 9-12, 2024; Florida, USA.
8. Chew EY, Clemons TE, Jaffe GJ, et al. Effect of ciliary neurotrophic factor on retinal neurodegeneration in patients with macular telangiectasia type 2: A randomized clinical trial. Ophthalmology. 2019; 126(4): 540-549.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	07.25.24	11.24
Drug is now FDA approved – criteria updated per FDA labeling: revised diagnostic criteria from one of the following to fluorescein leakage and one other feature to better align with pivotal study design; revised age requirement from age ≥ 21 years to ≥ 18 years; added requirements for BCVA of 54 letters or better on ETDRS (20/80 Snellen) and for no evidence of neovascularization in alignment with pivotal study design and as supported by retina specialist feedback; references reviewed and updated.	04.08.25	05.25

Important Reminder
This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical

policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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