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Revakinagene taroretcel-lwey

Table of Contents
Coverage
Policy Guidelines
Description
Rationale
Coding
References
Policy History

Related Policies (if applicable)
None

Disclaimer

Medical policies are a set of written guidelines that support current standards of practice. They are based on current generally accepted standards of care developed by: nonprofit professional association(s) for the relevant clinical specialty, third-party entities that develop treatment criteria, or other federal or state governmental agencies. A requested therapy must be proven effective for the relevant diagnosis or procedure. For drug therapy, the proposed dose, frequency and duration of therapy must be consistent with recommendations in at least one authoritative source. This medical policy is supported by FDA-approved labeling and/or nationally recognized authoritative references to major drug compendia, peer reviewed scientific literature and generally accepted standards of medical care. These references include, but are not limited to: MCG care guidelines, DrugDex (IIa level of evidence or higher), NCCN Guidelines (IIb level of evidence or higher), NCCN Compendia (IIb level of evidence or higher), professional society guidelines, and CMS coverage policy.

Carefully check state regulations and/or the member contract.

Each benefit plan, summary plan description or contract defines which services are covered, which services are excluded, and which services are subject to dollar caps or other limitations, conditions or exclusions. Members and their providers have the responsibility for consulting the member's benefit plan, summary plan description or contract to determine if there are any exclusions or other benefit limitations applicable to this service or supply. **If there is a discrepancy between a Medical Policy and a member's benefit plan, summary plan description or contract, the benefit plan, summary plan description or contract will govern.**

Legislative Mandates

EXCEPTION: For members residing in the state of Ohio, § 3923.60 requires any group or individual policy (Small, Mid-Market, Large Groups, Municipalities/Counties/Schools, State Employees, Fully-Insured, PPO, HMO, POS, EPO) that covers prescription drugs to provide for the coverage of any drug approved by the U. S. Food and Drug Administration (FDA) when it is prescribed for a use recognized as safe and effective for the treatment of a given indication in one or more of the standard medical reference compendia adopted by the United States Department of Health and Human Services or in medical literature even if the FDA has not approved the drug for that indication. Medical literature support is only satisfied when safety and efficacy has been confirmed in two articles from major peer-reviewed professional medical journals that present data supporting the proposed off-label use or uses as generally safe and effective. Examples of accepted journals include, but are not limited to, Journal of

American Medical Association (JAMA), New England Journal of Medicine (NEJM), and Lancet. Accepted study designs may include, but are not limited to, randomized, double blind, placebo controlled clinical trials. Evidence limited to case studies or case series is not sufficient to meet the standard of this criterion. Coverage is never required where the FDA has recognized a use to be contraindicated, and coverage is not required for non-formulary drugs.

EXCEPTION: For members residing in the state of Arkansas, § 23-79-147 relating to off-label prescription drug coverage, requires any plan that covers prescription drugs to provide for the coverage of any anticancer chemotherapeutic regimen ("drug") approved by the U.S. Food and Drug Administration (FDA) when that drug has been recognized as safe and effective for treatment of that specific type of cancer in standard reference compendia, (the American Hospital Formulary Service Drug Information, the National Comprehensive Cancer Network Drugs and Biologics Compendium, The Elsevier Gold Standard's Clinical Pharmacology), or has been recognized as safe and effective for treatment of that specific type of cancer in two [2] articles from medical literature that have not had their recognition of the drug's safety and effectiveness contradicted by clear and convincing evidence presented in another article from medical literature, or other authoritative compendia as identified by the Secretary of the United States Department of Health and Human Services. This coverage must include medically necessary services associated with the administration of the drug, provided that such services are covered by the insurance policy. However, coverage is never required where the FDA has recognized a use to be contraindicated. This applies to the following: Fully Insured Group, Student, Small Group, Mid-Market, Large Group, HMO, EPO, PPO, POS. Unless indicated by the group, this mandate or coverage will not apply to ASO groups.

Coverage

Revakinagene taroretcel-lwey (Encelto™) **may be considered medically necessary** for adults with idiopathic macular telangiectasia (MacTel) type 2 when the following criteria are met:

- The individual has no ocular or periocular infection;
- The individual does not have neovascular macular telangiectasia;
- The individual has a submitted photoreceptor inner segment/outer segment (IS/OS PR) break (loss) in ellipsoid zone (EZ) between 0.16 and 2.00 mm^2 measured by spectral domain optical coherence tomography (SD-OCT);
- The individual has a best corrected visual acuity (BCVA) of 54-letter score or better (20/80) as measured by the Early Treatment Diabetic Retinopathy Study (ETDRS) chart at screening.

Revakinagene taroretcel-lwey (Encelto™) **is considered experimental, investigational and/or unproven** for all non-U.S. Food and Drug Administration (FDA) approved indications.

Policy Guidelines

Revakinagene taroretcel-lwey (Encelto™) is administered by a single surgical intravitreal procedure performed by a qualified ophthalmologist.

Description

Macular telangiectasia (MacTel) is a disease affecting the macula of the eye, causing loss of central vision. It develops when there are problems with tiny blood vessels around the fovea, in the center of the macula, that provides the sharpest central vision for activities such as reading. (2)

MacTel Type 2, or idiopathic macular telangiectasia occurs when the tiny blood vessels around the fovea become abnormal and dilate. There is no known cause for this; and in some instances, new blood vessels form under the retina, which is called macular neovascularization. These blood vessels leak fluid or bleed, and that fluid causes the macula to swell or thicken affecting central vision. Type 2 affects both eyes but not always with the same severity. (2)

There are no symptoms in early stages of MacTel. Over time, individuals may experience blurring, distorted vision and loss of central vision. Brighter lights are needed to read or perform other functions. Loss of central vision progresses over a period of 10 to 20 years. It does not affect peripheral vision and does not usually cause total blindness. Type 2 MacTel is most commonly found in middle-aged adults, affecting men and women equally. Those with diabetes or hypertension may be at increased risk. There may be a genetic predisposition as this tends to run in families. (2)

During routine eye exams, small fine crystals may be found in the center of the macula. There may also be discoloration of the macula, abnormal blood vessels in the center of the macula, lipid or fat deposits and pigment clumps. Optical coherence tomography (OCT), OCT angiography (OCTA) and fluorescein angiography (FA) may be used for diagnosis. (2)

Regulatory Status
In March 2025, the U.S. Food and Drug Administration (FDA) approved revakinagene taroretcel-lwey (Encelto™; Neurotech Pharmaceuticals, Inc., Cumberland, RI) intravitreal implant for the treatment of adults with idiopathic macular telangiectasia type 2 (MacTel). (1)

Rationale

This policy is based on the U.S. Food and Drug Administration label for revakinagene taroretcel-lwey (Encelto™).

Revakinagene taroretcel-lwey (Encelto™) (1)
The efficacy of Encelto was evaluated in two studies, Study NTMT-03-A (NCT03316300; Study 1) 387 and Study NTMT-03-B (NCT03319849; Study 2) as described below.

Study 1
Study 1 was a randomized, multi-center, sham-controlled study which enrolled adults with MacTel. For enrollment, the patients were required to have a photoreceptor inner segment/ outer segment (IS/OS PR) break (loss) in ellipsoid zone (EZ) between 0.16 and 2.00 mm^2

measured by spectral domain-optical coherence tomography (SD-OCT) and best corrected visual acuity (BCVA) of 54-letter score or better (20/80 or better) as measured by the Early Treatment Diabetic Retinopathy Study (ETDRS) chart at screening. Patients with neovascular MacTel were excluded. Patients were randomized to receive either Encelto intravitreal implant or sham procedure under standard operative procedures. Patients in the Encelto group underwent conjunctival peritomy, implant placement in the vitreous cavity via sclerotomy and closure with sutures. Patients in the Sham group underwent conjunctival peritomy, scleral pressure, and conjunctival closure with sutures. One hundred and fifteen (96%) of 120 patients underwent the assigned procedure and were included in the analysis of efficacy.

A total of 120 patients were randomized and of these, 115 patients (Encelto group: 58; Sham group: 57) comprise the efficacy analysis population. The demographic characteristics of the efficacy analysis population were as follows: the mean age was 61 years (range 40 to 78 years), 79 patients (69%) were female, 98 patients (85%) were White, 5 patients (4%) were Asian, 3 patients (3%) were Black or African American, 1 patient (1%) was American Indian, and 8 patients (7%) were of "other" race. Six patients (5%) were Hispanic. The median (min, max) baseline EZ area was 0.35 (0.15, 1.99) mm^2 for the Encelto group and 0.36 (0.16, 1.7) mm^2 for the Sham group. The median (min, max) baseline aggregate sensitivity of microperimetry within the EZ break area 35.2 (0.75, 398.8) decibels (dB) for the Encelto group and 35.5 (2, 281.3) dB for the Sham group.

The primary efficacy outcome measure was the rate of change in the area of EZ loss (IS/OS, macular PR loss) over 24 months, as measured by SD-OCT. The secondary outcome measure was the mean change in aggregate sensitivity loss of microperimetry within the EZ break area from baseline to Month 24.

The efficacy outcome results for Study 1 are summarized in Table 1.

Table 1. Efficacy Results for Study 1 (N=115)

Efficacy Endpoints	**Encelto n=58**	**Sham n=57**	**Difference Encelto-Sham**	**P-value[c]**
Rate of change in EZ area loss from baseline over 24 months[a]				
mm^2	0.075	0.166	-0.091	<0.0001
(95% CI)	(0.05, 0.10)	(0.14, 0.19)	(-0.13, -0.06)	
Mean change in aggregate retinal sensitivity loss from baseline to 24 months[b]				
dB	25.27	43.02	-17.75	0.02
(95% CI)	(15.88, 34.67)	(37.78, 54.26)	(-32.58, -2.91)	

CI: confidence interval; dB: decibels; EZ: ellipsoid zone; mm: millimeters.

[a] Estimated by using a longitudinal mixed model including EZ area loss as the dependent variable, patient-specific random intercepts, treatment group, time (continuous), and interaction between treatment and time as covariates. The baseline and Months 12, 16, 20, and 24 visits were included.
[b] Estimated by using two-sample t-test; seven ENCELTO and four Sham patients were excluded due to missing data.
[c] Statistically significant at two-sided alpha of 0.05.

Study 2

Study 2 was a randomized, multi-center, sham-controlled study which enrolled adult with MacTel. For enrollment, the patients were required to have an IS/OS PR break in EZ between 0.16 and 2.00 mm^2 measured by SD-OCT and BCVA of 54-letter score or better (20/80 or better) as measured by the ETDRS chart at screening. Patients with neovascular MacTel were excluded.

Patients were randomized to receive either Encelto intravitreal implant or sham procedure under standard peri-operative procedures. Patients in Encelto group underwent conjunctival peritomy, implant placement in the vitreous cavity via sclerotomy and closure with sutures. Patients in the Sham group underwent conjunctival peritomy, scleral pressure, and conjunctival closure with sutures. One hundred and thirteen (95%) of the 119 patients underwent the assigned procedure and were included in efficacy evaluation.

A total of 119 patients were randomized and of these, 113 patients (Encelto group: 59; Sham group: 54) comprise the efficacy analysis population. The demographic characteristics of the efficacy analysis population were as follows: the mean age was 59 years (range: 40 to 75 years), 82 patients (73%) were female, 102 patients (90%) were White, 4 patients (4%) were Asian, and 7 patients (6%) were of "other" race or "unable to specify" race. Eight patients (7%) were Hispanic. The median (min, max) baseline EZ area was 0.48 (0.16, 1.63) mm^2 for the Encelto and 0.39 (0.16, 1.38) mm^2 for the Sham group. The median (min, max) baseline aggregate sensitivity of microperimetry within the EZ break area 40.07 (4.82, 291.52) dB for the Encelto group and 28.86 (0.33, 221.17) dB for the Sham group.

The primary efficacy outcome measure was the rate of change in the area of EZ loss (IS/OS, macular PR loss) over 24 months, as measured by SD-OCT. The secondary outcome measure was the mean change in aggregate sensitivity loss of microperimetry within the EZ break area from baseline to Month 24.

The efficacy results from Study 2 are summarized in Table 2 below.

Table 2. Efficacy Results for Study 2 (N=113)

Efficacy Endpoints	**Encelto n=59**	**Sham n=54**	**Difference Encelto-Sham**	**P-value[c]**
Rate of change in EZ area loss from baseline over 24 months[a] mm^2	0.111	0.160	-0.049	0.0186[c]

(95% CI)	(0.08, 0.14)	(0.13, 0.19)	(-0.089, -0.008)	
Mean change in aggregate retinal sensitivity loss from baseline to 24 months[b] dB (95% CI)	 40.02 (26.08, 53.96)	 41.97 (30.34, 53.60)	 -1.95 (-20.33, 16.43)	 0.83

CI: confidence interval; dB: decibels; EZ: ellipsoid zone; mm: millimeters.
[a] Estimated by using a longitudinal mixed model including EZ area loss as the dependent variable, patient-specific random intercepts, treatment group, time (continuous), and interaction between treatment and time as covariates. The baseline and Months 12, 16, 20, and 24 visits were included.
[b] Estimated by using two-sample t-test; Seven Encelto and six Sham patients were excluded due to missing data.
[c] Statistically significant at two-sided alpha of 0.05.

According to the FDA label, the safety and effectiveness of Encelto have not been established in pediatric patients.

Coding

Procedure codes on Medical Policy documents are included **only** as a general reference tool for each policy. **They may not be all-inclusive.**

The presence or absence of procedure, service, supply, or device codes in a Medical Policy document has no relevance for determination of benefit coverage for members or reimbursement for providers. **Only the written coverage position in a Medical Policy should be used for such determinations.**

Benefit coverage determinations based on written Medical Policy coverage positions must include review of the member's benefit contract or Summary Plan Description (SPD) for defined coverage vs. non-coverage, benefit exclusions, and benefit limitations such as dollar or duration caps.

CPT Codes	None
HCPCS Codes	J3403

*Current Procedural Terminology (CPT®) ©2025 American Medical Association: Chicago, IL.

References

U.S. Food and Drug Administration Label:

1. FDA. Highlights of Prescribing Information Encelto™ (revakinagene taroretcel-lwey). Revised March 2025. Available at: <https://www.accessdata.fda.gov> (accessed January 8, 2026).

Other:

2. Turbert D. What is Macular Telangiectasia? Available at: <https://www.aao.org> (accessed January 13, 2026).

Centers for Medicare and Medicaid Services (CMS)

The information contained in this section is for informational purposes only. HCSC makes no representation as to the accuracy of this information. It is not to be used for claims adjudication for HCSC Plans.

The Centers for Medicare and Medicaid Services (CMS) does not have a national Medicare coverage position. Coverage may be subject to local carrier discretion.

A national coverage position for Medicare may have been developed since this medical policy document was written. See Medicare's National Coverage at <https://www.cms.hhs.gov>.

Policy History/Revision

Date	Description of Change
06/01/2026	New medical document. Revakinagene taroretcel-lwey (Encelto™) may be considered medically necessary for adults with idiopathic macular telangiectasia (MacTel) type 2 when the following criteria are met: The individual has no ocular or periocular infection; The individual does not have neovascular macular telangiectasia; The individual has a submitted photoreceptor inner segment/outer segment (IS/OS/PR) break (loss) in ellipsoid zone (EZ) between 0.16 and 2.00 mm^2 measured by spectral domain optical coherence tomography (SD-OCT); The individual has a best corrected visual acuity (BCVA) of 54-letter score or better (20/80) as measured by the Early Treatment Diabetic Retinopathy Study (ETDRS) chart at screening. Revakinagene taroretcel-lwey (Encelto™) is considered experimental, investigational and/or unproven for all non-U.S. Food and Drug Administration (FDA) approved indications.