Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy

CENTENE® Corporation

Clinical Policy: Idebenone (Brand Name)

Reference Number: CP.PHAR.752
Effective Date: FDA Approval Date
Last Review Date: 11.25
Line of Business: Commercial, HIM, Medicaid

Revision Log

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

Description

Idebenone (Brand Name®/™) is a synthetic short-chain benzoquinone and coenzyme Q10 analogue that functions as a mitochondrial electron carrier and antioxidant.

FDA Approved Indication(s) [Pending]

Idebenone is indicated for the treatment of visual impairment in adolescent and adult patients with Leber's hereditary optic neuropathy (LHON) who carry a confirmed pathogenic mitochondrial DNA mutation affecting the ND1, ND4, or ND6 subunits of complex 1.

Limitation(s) of use: [Pending]

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.

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

I. Initial Approval Criteria*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Leber's Hereditary Optic Neuropathy (must meet all)**:**

1. Diagnosis of LHON;*
2. Genetic testing confirms the presence of one of the following (a, b, or c):*
   a. m.11778G>A mutation in the MT-ND4 gene;
   b. m.3460G>A mutation in the MT-ND1 gene;
   c. m.14484T>C mutation in the MT-ND6 gene;
3. Prescribed by or in consultation with an ophthalmologist;
4. Age ≥ 12 years;*
5. Documentation of member's baseline visual acuity (VA) using the Early Treatment of Diabetic Retinopathy Study (ETDRS) chart;*
6. Member has impaired VA in at least one eye due to LHON;*
7. Onset of vision loss due to LHON occurred within the last 5 years;*
8. Dose does not exceed 900 mg (6 tablets) per day.*

Approval duration: 12 months

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*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Leber's Hereditary Optic Neuropathy (must meet all)**:**
1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B)*;
2. Member is responding positively to therapy as evidenced by one of the following (a or b):*
   a. For the first re-authorization request, one of the following (i, ii, or iii):
      i. If member had an off-chart VA (i.e., unable to read any letter on the ETDRS chart) at baseline, improvement of VA to reading at least 5 letters on the ETDRS chart (i.e., VA is ≤ 1.6 logMAR);
      ii. If member had an on-chart VA (i.e., able to read letters on the ETDRS chart) at baseline, improvement of VA by at least 10 additional letters (i.e., change of -0.2 logMAR) on the ETDRS chart;
      iii. Maintenance of VA from baseline without deterioration to legal blindness (i.e., VA remains < 1.0 logMAR) at the most recent visit on the ETDRS chart;
   b. For second or subsequent re-authorization requests: Improvement or maintenance of VA from the last request at the most recent visit on the ETDRS chart;
3. If request is for a dose increase, new dose does not exceed 900 mg (6 tablets) per day.*

Approval duration: 12 months

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

FDA: Food and Drug Administration
LHON: Leber's hereditary optic neuropathy
ETDRS: Early Treatment Diabetic Retinopathy Study
VA: visual acuity

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

- Contraindication(s): pending
- Boxed warning(s): pending

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
LHON *	300 mg PO TID*	900 mg/day*

VI. Product Availability [Pending]

Tablet: 150 mg*

VII. References

1. European Medicines Agency. Raxone (idebenone): EPAR – Product Information; August 2025. Available at: https://www.ema.europa.eu/en/medicines/human/EPAR/raxone. Accessed October 6, 2025.
2. Klopstock T, Yu-Wai-Man P, Dimitriadis K, et al. A randomized placebo-controlled trial of idebenone in Leber's hereditary optic neuropathy. Brain. 2011;134(Pt 9):2677-2686. doi:10.1093/brain/awr170
3. Yu-Wai-Man P, Carelli V, Newman NJ, et al. Therapeutic benefit of idebenone in patients with Leber hereditary optic neuropathy: The LEROS nonrandomized controlled trial. Cell Rep Med. 2024;5(3):101437. doi:10.1016/j.xcrm.2024.101437
4. Carelli V, Carbonelli M, de Coo IF, et al. International consensus statement on the clinical and therapeutic management of Leber hereditary optic neuropathy. J Neuroophthalmol. 2017;37(4):371-381. doi:10.1097/WNO.0000000000000570

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	10.21.25	11.25

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.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

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.