Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy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: Sonpiretigene Isteparvovec (MCO-010)

Reference Number: CP.PHAR.749
Effective Date: FDA Approval Date
Last Review Date: 11.25
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

Sonpiretigene isteparvovec (MCO-010®/™) is an adeno-associated virus serotype 2 (AAV2) gene therapy.

FDA Approved Indication(s) [Pending]

MCO-010 is indicated for the treatment of adult patients with advanced retinitis pigmentosa (RP) with severe vision loss.

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 MCO-010 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. Retinitis Pigmentosa (must meet all)**:**

1. Diagnosis of RP as confirmed by genetic testing;*
2. Prescribed by an ophthalmologist or retina specialist;*
3. Age ≥ 18 years;*
4. Member has significant vision loss as evidenced by both of the following (a and b; *see Appendix D*):*
   a. Best-corrected visual acuity (BCVA) worse than 1.9 logMAR in the eye receiving MCO-010;
   b. BCVA less than 1.6 logMAR in the eye not receiving treatment;
5. Provider attestation that treatment with MCO-010 will only be given in the eye with the lowest visual acuity;*
6. Member has not previously been treated with MCO-010;*
7. Dose does not exceed FDA maximum dose.*

Approval duration: 4 weeks (1 lifetime dose)

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. Retinitis Pigmentosa
1. Continued therapy will not be authorized as MCO-010 is indicated to be a one-time application per lifetime.*

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
FDA: Food and Drug Administration
LogMAR: logarithmic minimum angle of resolution
RP: retinitis pigmentosa
VA: visual acuity

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

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

Appendix D: General Information

- Significant vision loss as evidenced by visual acuity (VA) description:
  - BCVA is a validated measure of acuity that evaluates the best vision achievable using corrected lenses. It is commonly used in clinical practice and clinical trials. BCVA is typically assessed by having individuals identify letters of varying size on a chart.
  - Freiberg Visual Acuity and Contrast Test (FrACT) is a validated measure of VA. This tool displays aids such as a letter displayed at varying sizes and orientations for the individual to identify to determine their visual acuity. FrACT can assess individuals with very low vision to the range of semiquantitative categories of "counting fingers" (equivalent to approximately 1.9 logMAR) and even "hand motion" (approximately 2.3 logMAR).
  - LogMAR is a unit of measure of VA ranging from -0.3 to 2.25 for the FrACT test used in the RESTORE trial. A LogMAR of zero corresponds to 20/20 vision with values increasing above zero indicating worsening visual acuity. In the RESTORE trial, an improvement by at least -0.3 LogMAR, or three lines gained, is considered clinically meaningful.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
RP*	Pending*	Pending*

VI. Product Availability [Pending]

Pending*

VII. References

1. ClinicalTrials.gov. Efficacy and safety of MCO-010 Optogenetic therapy in adults with retinitis pigmentosa (RESTORE). Available at: https://clinicaltrials.gov/study/NCT04945772. Accessed August 20, 2025.

2. Institute for Clinical and Economic Review. Sonpiretigene isteparvovec for advanced retinitis pigmentosa: Effectiveness and value (final report). Published May 15, 2025. Available at: https://icer.org/wp-content/uploads/2025/05/ICER_RP_Final-Report_For-Publication_051525.pdf. Accessed August 20, 2025.
3. Institute for Clinical and Economic Review. Report at a glance: Retinitis pigmentosa. Published May 2025. Available at: https://icer.org/wp-content/uploads/2025/05/RP_RAAG_May-2025.pdf. Accessed August 20, 2025.
4. Nguyen XT, Moekotte L, Plomp AS, et al. Retinitis pigmentosa: Current clinical management and emerging therapies. Int J Mol Sci. 2023 Apr 19;24(8):7481. doi: 10.3390/ijms24087481.

Coding Implications [Pending]
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
Pending	Pending

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	08.26.25	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.