Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy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: Ifezuntirgene Inilparvovec (AMT-130)

Reference Number: CP.PHAR.761
Effective Date: FDA Approval Date
Last Review Date: 02.26
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

Ifezuntirgene inilparvovec (AMT-130) is an adeno-associated virus (AAV5) vector based gene therapy designed to inhibit the production of the mutant huntingtin protein (mHTT).

FDA Approved Indication(s) [Pending]

AMT-130 is indicated for the treatment of Huntington's disease (HD).

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 Precision Drug Action Committee (PDAC) Utilization Management Review**. Refer to CC.PHAR.21 for process details.

It is the policy of health plans affiliated with Centene Corporation® that AMT-130 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. Huntington's Disease (must meet all)**:**

1. Diagnosis of HD;*
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 25 years;*
4. Targeted mutation analysis demonstrates a cytosine-adenine-guanine (CAG) trinucleotide expansion of ≥ 40 repeats in the huntingtin (HTT) gene;
5. Recent (within the last 60 days) Unified Huntington's Disease Rating Scale (UHDRS) assessment with both of the following (a and b):
   a. Total Functional Capacity (TFC) score ≥ 9;
   b. Diagnostic Confidence Level (DCL) of one of the following (i or ii):
      i. Score of 4 (motor abnormalities that are unequivocal signs of HD);
      ii. Score of 3 (motor abnormalities that are likely signs of HD) and one of the following (1, 2, or 3):
         1) Positive ('Yes') response to UHDRS Question 80 (multidimensional manifest diagnosis on motor, cognitive, behavioral, functional – *see Appendix D*);

2) Member satisfies the DSM5 criteria for cognitive disorder (Movement Disorder Society Task Force criteria – *see Appendix D*);
3) Member has cognitive symptoms consistent with HD (examples include but are not limited to difficulty with organization, planning, and prioritizing tasks, impaired judgment and decision-making, reduced concentration and attention, word-finding difficulties);

6. Member has no pre-existing antibodies to AAV5 as measured by an FDA-approved test;
7. Member has not received prior gene therapy;
8. Provider attestation that brain and spinal pathology were assessed and no pathology was identified that may interfere with the surgical delivery of AMT-130 or represents a significant neurologic comorbid disorder;
9. Dose does not exceed a single infusion of 6 x 10^{13} genome copies.

Approval duration: 3 months (*1 dose only*)

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. Huntington's Disease

1. Continued therapy will not be authorized as AMT-130 is indicated to be dosed one time only.

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

AAV: adeno-associated virus
CAG: cytosine-adenine-guanine
DCL: diagnostic confidence level
FDA: Food and Drug Administration
HD: Huntington's disease
HTT: huntingtin
m: mutant
TFC: total functional capacity
UHDRS: Unified Huntington's Disease Rating Scale

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

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

Appendix D: General Information

- UHDRS Question 80 is a single yes/no question that asks the examiner the following: Based on the entire UHDRS (Motor, Cognitive, Behavioral and Functional components), do you believe with a confidence level ≥ 99% that this participant has manifest HD?
- For Huntington's Disease diagnosis, the Movement Disorder Society (MDS) Task Force recommendations integrate the presence of neurocognitive disorder with the motor criteria, assessing for either of the following:
  - Major Neurocognitive Disorder (Dementia): Uses standard DSM-5 criteria, requiring evidence of significant cognitive decline in one or more domains that interferes with independence in everyday activities.
  - Mild Neurocognitive Disorder: Uses standard DSM-5 criteria, requiring evidence of modest cognitive decline in one or more domains that does not interfere with independence in everyday activities, though greater effort may be required.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
HD*	6×10^{13} genome copies as a single MRI-guided stereotactic infusion*	6×10^{13} genome copies*

VI. Product Availability [Pending]

Pending*

VII. References

1. National Library of Medicine. Safety and proof-of-concept (POC) study with AMT-130 in adults with early manifest Huntington's disease. ClinicalTrials.gov ID NCT04120493. Available at: https://www.clinicaltrials.gov/study/NCT04120493. Accessed November 25, 2025.
2. Ross CA, Reilmann R, Cardoso F, et al. Movement Disorder Society Task Force Viewpoint: Huntington's disease diagnostic categories. Mov Disord Clin Pract. 2019 Aug 23;6(7):541-546. doi: 10.1002/mdc3.12808.
3. Press Release: uniQure announces positive topline results from pivotal phase I/II study of AMT-130 in patients with Huntington's disease. September 24, 2025. Available at: https://uniqure-production-fe68e1a2.preview.craft.cloud/investors-media/press-releases. Accessed November 25, 2025.
4. Press Release: uniQure provides regulatory update on AMT-130 for Huntington's disease. November 3, 2025. Available at: https://uniqure-production-fe68e1a2.preview.craft.cloud/investors-media/press-releases. Accessed November 25, 2025.
5. Trundell D, Palermo G, Schobel S, et al. Defining clinically meaningful change on the Composite Unified Huntington's Disease Rating Scale (cUHDRS). Neurology. April 9, 2019. 92(15_supplement): P1.8-043.
6. Bean L, Bayrak-Toydemir P, ACMG Laboratory Quality Assurance Committee. Addendum: American College of Medical Genetics and Genomics Standards and Guidelines for Clinical Genetics Laboratories, 2014 edition: technical standards and guidelines for Huntington disease. Genet Med. 2021;23(12):2461.
7. Kremer B, Goldberg P, Andrew SE. A worldwide study of the Huntington's disease mutation: the sensitivity and specificity of measure CAG repeats. NEJM. May 19, 1994; 330(20):1401-1406.
8. Unified Huntington's disease rating scale: reliability and consistency. Movement Disorder Society. Movement Disorders. 1996;11(2):136-143.
9. Mestre TA, Forjaz MJ, Mahlknecht P, et al. Rating scales for motor symptoms and signs in Huntington's disease: Critique and recommendation. International Parkinson and Movement Disorders Society. Movement Disorders Clinical Practice. 2018;5(2):111-117. DOI:10.1002/mdc3.1257.

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	12.02.25	02.26

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.