

Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy



## Clinical Policy: Adrabetadex (VTS-270)

Reference Number: CP.PHAR.777

Effective Date: **FDA Approval Date**

Last Review Date: 05.26

Line of Business: Commercial, HIM/ICHRA, Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

### Description

Adrabetadex (VTS-270) is a mixture of cyclic oligosaccharides.

### FDA Approved Indication(s) [Pending]

VTS-270 is indicated for the treatment of infantile-onset Niemann-Pick disease type C (NPC).

### 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 VTS-270 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. Niemann-Pick Disease Type C (must meet all):

1. Diagnosis of NPC confirmed by one of the following (a or b):
  - a. Genetic analysis indicating mutation in both alleles of *NPC1* or *NPC2*;
  - b. Genetic analysis indicating mutation in one allele of *NPC1* or *NPC2* along with one of the following (i or ii):
    - i. Positive filipin staining test result;
    - ii. Positive biomarker result (e.g., oxysterol, lyso-sphingolipid, bile acid);
2. Prescribed by or in consultation with a geneticist, neurologist, endocrinologist, or metabolic disease specialist;
3. Member presents with both of the following (a and b):\*
  - a. At least one neurological sign or symptom of the disease (*see Appendix D*);
  - b. Neurological sign/symptom onset < 6 years of age (i.e., infantile-onset);
4. Adrabetadex is not prescribed concurrently with Aqneursa™ or Miplyffa™;\*
5. Dose does not exceed 900 mg intrathecally every 2 weeks.\*

##### Approval duration:

**Medicaid/HIM/ICHRA** – 12 months

**Commercial** – 6 months or to the member's renewal date, whichever is longer

**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/ICHRA) 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/ICHRA, and CP.PMN.255 for Medicaid; or
  - b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) 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/ICHRA, 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/ICHRA, and CP.PMN.53 for Medicaid.

**II. Continued Therapy\***

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

**A. Niemann-Pick Disease Type C** (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 an improvement, stabilization, or slowed disease progression in a domain affected by NPC (e.g., ambulation, fine motor skills, swallowing, sitting, speech);\*
3. Adrabetadex is not prescribed concurrently with Aqneursa or Miplyffa;\*
4. If request is for a dose increase, new dose does not exceed 900 mg intrathecally every 2 weeks.\*

**Approval duration:**

**Medicaid/HIM/ICHRA** – 12 months

**Commercial** – 6 months or to the member’s renewal date, whichever is longer

**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/ICHRA) 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/ICHRA, and CP.PMN.255 for Medicaid; or
  - b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) 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/ICHRA, and CP.PMN.16 for Medicaid; or
- 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/ICHRA, and CP.PMN.53 for Medicaid.

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

- 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/ICHRA, 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

NPC: Niemann-Pick disease type C

*Appendix B: Therapeutic Alternatives*

Not applicable

*Appendix C: Contraindications/Boxed Warnings [Pending]*

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

*Appendix D: General Information*

Examples of neurological signs or symptoms of NPC include hearing loss, vertical supranuclear gaze palsy, delayed developmental motor milestones, developmental delay/regression, dysarthria, ataxia, dystonia, impaired ambulation, dysarthria, dysphagia, seizures, and dementia.

**V. Dosage and Administration [Pending]**

Indication	Dosing Regimen	Maximum Dose
NPC*	Intrathecal; pending	900 mg/2 weeks*

**VI. Product Availability [Pending]**

Single-dose vial for injection: 900 mg/4.5 mL\*

**VII. References**

- 54th Child Neurology Society Annual Meeting. *Ann Neurol.* 2025;98 Suppl 35:S4-S170.
- ClinicalTrials.gov. VTS-270 to treat Niemann-Pick type C1 (NPC1) disease. Available at: <https://clinicaltrials.gov/study/NCT02534844>. Accessed March 26, 2026.
- Beren Therapeutics. Beren Therapeutics presents data supporting the efficacy of adrabetadex in infantile-onset NPC at 2026 ACMG Clinical Genetics Meeting. March 12, 2026. Available at: <https://www.berentx.com/news/beren-therapeutics-presents-data-supporting-the-efficacy->

of-adrabetadex-in-infantile-onset-npc-at-2026-acmg-clinical-genetics-meeting. Accessed March 26, 2026.

4. Geberhiwot T, Moro Alessandro, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. Orphanet Journal of Rare Diseases 2018 April 6;13(1):50.
5. Patterson MC, Clayton P, Gissen P, et al. Recommendations for the detection and diagnosis of Niemann-Pick disease type C: An update. Neurol Clin Pract. 2017;7(6):499-511.

**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	03.31.26	05.26

**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.

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