

Clinical Policy: Eladocagene Exuparvovec-tneq (Kebilidi)

Reference Number: CP.PHAR.595

Effective Date: 11.13.24

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

Eladocagene exuparvovec-tneq (Kebilidi™) is a recombinant serotype 2 adeno-associated virus (rAAV2) based gene therapy designed to deliver a copy of the dopa decarboxylase (*DDC*) gene which encodes the aromatic L-amino acid decarboxylase (AADC) enzyme.

FDA Approved Indication(s)

Kebilidi is indicated for the treatment of adults and pediatric patients with AADC deficiency.

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 Kebilidi is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. AADC Deficiency (must meet all):

1. Diagnosis of AADC deficiency as evidenced by documentation of positive testing from two of the following core diagnostic tests (see *Appendix E*):
 - a. Cerebrospinal fluid (CSF) neurotransmitter metabolite panel;
 - b. Single gene or genetic panel testing;
 - c. Plasma enzyme assay;
2. Prescribed by or in consultation with a geneticist or neurologist;
3. Age \geq 16 months and $<$ 11 years;
4. Evidence of classic clinical symptoms of AADC deficiency (e.g., hypotonia, dystonia, oculogyric crisis, unable to stand, developmental retardation, see *Appendix D*);
5. Member is unable to ambulate independently with or without assistive device;
6. Documentation that member has achieved skull maturity by neuroimaging;
7. Documentation of baseline laboratory tests demonstrating anti-AAV2 neutralizing antibody titer does not exceed $>$ 1,200 fold or ELISA optical density (OD) $>$ 1;
8. Member has not received prior gene therapy;
9. Dose does not exceed 1.8×10^{11} vg (0.32 mL total volume).

Approval duration: 3 months (one-time dose per lifetime)

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

A. AADC Deficiency

1. Continued therapy will not be authorized as Kebilidi 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

AADC: aromatic L-amino acid decarboxylase
CSF: cerebrospinal fluid
DDC: dopa decarboxylase
FDA: Food and Drug Administration

hAADC: human cDNA encoding the AADC enzyme
MAO: monoamine oxidase
OD: optical density
rAAV2: recombinant serotype 2 adeno-associated virus

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): patients who have not achieved skull maturity assessed by neuroimaging
- Boxed warning(s): none reported

Appendix D: General Information

- Classic clinical symptoms of AADC deficiency from 2017 Consensus guidelines for the diagnosis and treatment of AADC deficiency and AADC-010/AADC-011 inclusion criteria:
 - Movement disorders: hypotonia, dystonia, dyskinesia, tremor, myoclonus, oculogyric crisis, hypokinesia
 - Developmental delay: delayed motor development, delayed cognitive development, delayed speech development
 - Tone regulation: floppy infant, hypotonia, hypertonia, poor head control

Appendix E: Diagnostic Information

- Per 2017 consensus guideline for the diagnosis and treatment of AADC deficiency, there are three core diagnostic tools for identifying AADC deficiency. When feasible, it is recommended to conduct all three key diagnostic tests for patients:
 - Low CSF levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA), and 3-methoxy-4-hydroxyphenylglycol (MHPG), with normal CSF pterins, and increased CSF levels of L-dopa, 3-O-methyldopa (3-OMD), and 5-OH tryptophan (5-HTP)
 - Genetic diagnosis showing compounding heterozygous or homozygous disease causing variants in the *DDC* gene
 - Decreased AADC enzyme activity in plasma
- PTC Therapeutics' PTC Pinpoint Program has partnered with two companies, Invitae and MNG Laboratories, to offer no-cost testing.
 - The Invitae Neurotransmitter Disorders panel analyzes the *DDC* gene and analyzes for AADC deficiency. More information can be found on the Invitae website: <https://www.invitae.com/us/providers/test-catalog/test-06203>.

- MNG Laboratories offers blood testing for elevated levels of the neurotransmitter metabolite 3-OMD. If elevated levels of 3-OMD are present, more diagnostic tests will be completed, including AADC enzyme activity assessment and *DDC* gene sequencing. More information can be found on the website: <https://aadcinsights.com/no-cost-testing/>.
- At this time, the current evidence does not support medical necessity of Kebilidi for the treatment of AADC deficiency for patients ≥ 11 years and ambulatory patients for the following reasons:
 - No efficacy data was submitted for patients ≥ 11 year and ambulatory patients. FDA approval was based the safety and efficacy data from an open-label, single-arm clinical study, Study 1 (NCT04903288), in 13 pediatric patients (aged 1.3 to 10.8 years) who were unable to ambulate independently (with or without assistive devices).

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
AADC deficiency	Administer a total dose of 1.8×10^{11} vg (0.32 mL total volume) delivered as four 0.08 mL (0.45×10^{11} vg) intraputaminal infusions (two sites per putamen-anterior and posterior) at a rate of 0.003 mL/minute (0.18 mL/hour) for a total of 27 minutes per site	1.8×10^{11} vg (0.32 mL total volume)

VI. Product Availability

Single-dose vial for intraputaminal infusion: 2.8×10^{11} vg/0.5 mL (nominal concentration of 5.6×10^{11} vg/mL) of eladocagene exuparvovec-tneq and each 2 mL vial contains an extractable volume of 0.5 mL

VII. References

1. Kebilidi Prescriber Information. Warren, NJ. PTC Therapeutics, Inc; November 2024. Available at: <https://www.ptcbio.com/wp-content/uploads/sites/2/2024/11/Kebilidi-Prescribing-Information.pdf>. Accessed November 6, 2025.
2. Wassenberg T, Molero-Luis M, Jeltsch K, et al. Consensus guideline for the diagnosis and treatment of aromatic L-amino acid decarboxylase (AADC) deficiency. Orphanet J Rare Dis. 2017 Jan 18;12(1):12.
3. ClinicalTrials.gov. A clinical trial for treatment of aromatic L-amino acid decarboxylase (AADC) deficiency using AAV2-hAADC- an expansion. Available at: <https://clinicaltrials.gov/ct2/show/NCT02926066>. Accessed November 6, 2025.
4. ClinicalTrials.gov. A phase I/II clinical trial for treatment of aromatic L-amino acid decarboxylase (AADC) deficiency using AAV2-hAADC (AADC). Available at: <https://clinicaltrials.gov/ct2/show/NCT01395641>. Accessed November 6, 2025.
5. Clinical and economic data supporting formulary consideration of eladocagene exuparvovec. South Plainfield, NJ. PTC Therapeutics, Inc. September 2020.
6. Upstaza (eladocagene exuparvovec): Clinical trial data. PTC Therapeutics, Inc. July 2022.
7. Upstaza Product Information. Dublin, Ireland. PTC Therapeutics International Ltd. September 2022. Available at:

<https://www.ema.europa.eu/en/medicines/human/EPAR/upstaza>. Accessed November 20, 2025.

8. Golikeri A and Yi S. BLA clinical and clinical pharmacology review memorandum – Kebilidi. PTC Therapeutics, Inc. March 2024. Accessed November 20, 2025.

Coding Implications

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
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	09.27.22	11.22
4Q 2023 annual review: no significant changes as the drug is not yet FDA approved; references reviewed and updated.	07.18.23	11.23
4Q 2024 annual review: no significant changes as the drug is not yet FDA approved; for Appendix E, added supplemental information regarding PTC Therapeutic's Pinpoint Program that offers no-cost diagnosis testing; references reviewed and updated.	07.22.24	11.24
RT1: Drug is now FDA approved – criteria updated per FDA labeling: reduced the age requirement to 16 months to align with PI and pivotal study minimum age; added criterion “documentation that member has achieved skull maturity by neuroimaging” per PI; removed redirection to symptomatic relief therapies; updated FDA maximum dose and dosing regimen; added disclaimer “all requests reviewed under this policy require medical director review.”	01.14.25	02.25
Revised initial criteria to clarify anti-AAV2 neutralizing antibody titer “does not exceed > 1,200 fold.”	02.17.25	
Updated language under Policy/Criteria to effectively redirect prior authorization reviews to Precision Drug Action Committee (PDAC) Utilization Management Review.	11.04.25	
1Q 2026 annual review: restricted Kebilidi to age < 11 years and member is unable to ambulate independently with or without assistive device based on current available evidence; references reviewed and updated.	11.06.25	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.

©2022 Centene Corporation. All rights reserved. All materials are exclusively owned by Centene Corporation and are protected by United States copyright law and international copyright law. No part of this publication may be reproduced, copied, modified, distributed, displayed, stored in a retrieval system, transmitted in any form or by any means, or otherwise published without the prior written permission of Centene Corporation. You may not alter or remove any trademark, copyright or other notice contained herein. Centene® and Centene Corporation® are registered trademarks exclusively owned by Centene Corporation.