Clinical Policy: Eladocagene Exuparvovec-tneq (Kebilidi)

Reference Number: PA.CP.PHAR.595
Effective Date: 02/2025
Last Review Date: 01/2026

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 PA Health & Wellness® 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. Evidence of classic clinical symptoms of AADC deficiency (e.g., hypotonia, dystonia, oculogyric crisis, unable to stand, developmental retardation, see *Appendix D*);
4. Member is unable to ambulate independently with or without assistive device;
5. Documentation that member has achieved skull maturity by neuroimaging;
6. Documentation of baseline laboratory tests demonstrating anti-AAV2 neutralizing antibody titer does not exceed > 1,200 fold or ELISA optical density (OD) > 1;
7. Member has not received prior gene therapy;
8. 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

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. AADC Deficiency (must meet all)**:**

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. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

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 – PA.CP.PMN.53

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 decarboyxlase (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 July 19, 2024.
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.
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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
Policy created	01/2025
1Q 2026 annual review: Revised initial criteria to clarify anti-AAV2 neutralizing antibody titer “does not exceed > 1,200 fold.”; member is unable to ambulate independently with or without assistive device based on current available evidence; references reviewed and updated.	01/2026