Medical Policy

BlueCross BlueShield Kansas
An Independent licensee of the
Blue Cross Blue Shield Association

Title: Kebilidi (eladocagene exuparvovec-tneq) (Intraputaminal)

Professional / Institutional
Original Effective Date: March 27, 2025
Latest Review Date:
Current Effective Date: March 27, 2025

State and Federal mandates and health plan member contract language, including specific provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage. To verify a member's benefits, contact Blue Cross and Blue Shield of Kansas Customer Service.

The BCBSKS Medical Policies contained herein are for informational purposes and apply only to members who have health insurance through BCBSKS or who are covered by a self-insured group plan administered by BCBSKS. Medical Policy for FEP members is subject to FEP medical policy which may differ from BCBSKS Medical Policy.

The medical policies do not constitute medical advice or medical care. Treating health care providers are independent contractors and are neither employees nor agents of Blue Cross and Blue Shield of Kansas and are solely responsible for diagnosis, treatment and medical advice.

If your patient is covered under a different Blue Cross and Blue Shield plan, please refer to the Medical Policies of that plan.

POLICY AGENT SUMMARY – MEDICAL PRIOR AUTHORIZATION

Indication	Dose
AADC deficiency	– Total Recommended Dose: $1.8x10^{11}$ vg (0.32 mL) – Total number of infusions: 4 – Volume (dose) per infusion: 0.08 mL ($0.45x10^{11}$ vg) – Location of infusions: 2 in anterior putamen, 2 in posterior putamen – Infusion rate at each target point: 0.003 mL/min – Dose duration for infusion at each target point: 27 minutes

Indication	Dose
	• Kebilidi is administered using an infusion pump at a rate of 0.003 mL/min, infusion pump must be capable of infusing at this rate. • Kebilidi is administered as four intraputaminal infusions in a single stereotactic neurosurgical procedure. • **Do not** refreeze thawed product. • Kebilidi should be administered in a medical center which specializes in stereotactic neurosurgery. • Administer Kebilidi only using an FDA-authorized cannula for intraparenchymal infusion (i.e., ClearPoint SmartFlow Neuro Cannula).

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

I. Length of Authorization

Coverage will be provided for one dose and will not be renewed.

II. Dosing Limits

A. Max Units (per dose and over time) [HCPCS Unit]:

- $1.8x10^{11}$ vg (0.32 mL) one time only

III. Initial Approval Criteria [1-15]

– Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax.

Coverage is provided in the following conditions:

Aromatic L-amino acid decarboxylase (AADC) deficiency

- Patient is at least 16 months of age through 10 years of age; **AND**
- Patient has a diagnosis of severe Aromatic L-amino acid decarboxylase (AADC) deficiency as established by the following:
  - Patient has a biallelic pathogenic variants in *DDC* gene identified by molecular genetic testing; **OR**
  - Patient cerebrospinal fluid (CSF) or plasma neurotransmitter profile is consistent with AADC deficiency; **AND**
    - Patient has significantly reduced AADC enzyme activity in plasma.; **AND**
- Patient is experiencing persistent neurological defects (e.g., autonomic dysfunction, hypotonia, dystonia and other movement disorders, etc.) secondary to AADC deficiency despite standard medical therapy (e.g., dopamine agonists, monoamine oxidase inhibitor,

pyridoxine, or other forms of vitamin B6) Note: patients should be on stable dosages for at least 3 months prior to treatment with eladocagene; **AND**

- Patient is unable to ambulate independently; **AND**
- Patient has achieved skull maturity as assessed by neuroimaging; **AND**
- Patient does not have pyridoxine 5'-phosphate oxidase or tetrahydrobiopterin (BH4) deficiency; **AND**
- Patient has not received prior gene therapy; **AND**
- Patient must not have a baseline anti-AAV2 antibody titer above the established threshold for a positive result; **AND**
- Patient does not have any contraindications that would preclude the surgical intra-putaminal administration

† FDA Approved Indication(s); **‡** Compendia Recommended Indication(s); **Φ** Orphan Drug

IV. Renewal Criteria [1]

Coverage cannot be renewed.

Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

CLINICAL RATIONALE

See package insert for FDA preshttps://dailymed.nlm.nih.gov/dailymed/index.cfm

REFERENCES

1. Kebilidi [package insert]. Warren, NJ; PTC Therap., Inc., November 2024. Accessed November 2024.
2. ClinicalTrials.gov. NCT01395641. A Phase I/II Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC. https://clinicaltrials.gov/study/NCT01395641 .
3. ClinicalTrials.gov. NCT02926066. A Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC - An Expansion (NTUH-AADC-011). https://clinicaltrials.gov/study/NCT02926066 .
4. Blau N, Pearson TS, Kurian MA, et al. Aromatic L-Amino Acid Decarboxylase Deficiency. GeneReviews. https://www.ncbi.nlm.nih.gov/books/NBK595821/ (Accessed on August 5, 2024).
5. 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. https://doi.org/10.1186/s13023-016-0522-z.

CODING

The following codes for treatment and procedures applicable to this policy are included below for informational purposes. This may not be a comprehensive list of procedure codes applicable to this policy.

Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

The code(s) listed below are medically necessary ONLY if the procedure is performed according to the "Policy" section of this document.

HCPCS code:

- J3590 – Unclassified biologics

NDC:

- Kebilidi 5.6×10^{11} vector genomes (vg) per mL – 2 mL single dose vial: 52856-0601-xx

REVISIONS	
Posted 02-25-2025 Effective 03-27-2025	Policy added to the bcbsks.com web site. Policy maintained by Prime Therapeutics LLC.