Corporate Medical Policy: Eladocagene exuparvovec-tneq (Kebilidi®) **"Notification"** **POLICY EFFECTIVE APRIL 1, 2026**

Restricted Product(s):

- eladocagene exuparvovec-tneq (Kebilidi®) intraputaminal infusion for administration by a healthcare professional

FDA Approved Use:

- For the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency

Criteria for Medical Necessity:

The restricted product(s) may be considered medically necessary when the following criteria are met:

1. The patient is at least 16 months of age through 10 years of age; **AND**
2. The patient has a diagnosis of severe **aromatic L-amino acid decarboxylase (AADC) deficiency [medical record documentation required]**; **AND**
3. The patient's diagnosis has been confirmed by ALL of the following **[medical record documentation required]**:
    a. Molecular genetic testing demonstrating biallelic mutations in the human DOPA decarboxylase (*DDC*) gene **[medical record documentation required]**; **AND**
    b. Decreased plasma AADC enzyme activity **[medical record documentation required]**; **AND**
4. The patient is experiencing persistent neurological defects (e.g., autonomic dysfunction, hypotonia, movement disorders [oculogyric crises, dystonia, hypokinesia], developmental delay) secondary to AADC deficiency despite standard medical therapy (e.g., dopamine agonists, monoamine oxidase inhibitors, pyridoxine, or other forms of vitamin B6) on stable dosages for at least 3 months prior to treatment with the requested agent **[medical record documentation required]**; **AND**
5. The patient is unable to ambulate independently (with or without assistive device) **[medical record documentation required]**; **AND**
6. The patient has achieved skull maturity as assessed by neuroimaging **[medical record documentation required]**; **AND**
7. The patient does NOT have any other enzyme deficiencies, including pyridoxine 5'-phosphate oxidase or tetrahydrobiopterin (BH4) deficiency **[medical record documentation required]**; **AND**
8. The patient does NOT have a baseline anti-adeno-associated virus, serotype 2 (anti-AAV2) antibody titer higher than 1:1200 or greater than 1 optical density value by enzyme-linked immunosorbent assay **[medical record documentation required]**; **AND**
9. The patient does NOT have evidence of a clinically active infection **[medical record documentation required]**; **AND**
10. The patient has NOT received any previous gene therapy, including the requested agent **[medical record documentation required]**; **AND**

11. The prescriber is a specialist in the area of the patient's diagnosis (e.g., pediatric neurologist) or has consulted with a specialist in the area of the patient's diagnosis **[medical record documentation required]**; **AND**
12. The requested dose is within FDA labeled dosing for the requested indication, and the requested quantity does NOT exceed the maximum units allowed for the duration of approval (see table below) **[medical record documentation required]**.

Duration of Approval: 90 days (one-time treatment per lifetime)

Please note, for certain identified gene and cellular therapies such as eladocagene exuparvovec-tneq (Kebilidi®), when coverage is available and the individual meets medically necessary criteria, distribution from a specialty pharmacy provider due to cost (distribution channel restriction) may be required in order for coverage to be provided. **Please contact Blue Cross NC to coordinate this therapy.

FDA Label Reference				
Medication	**Indication**	**Dosing**	**HCPCS**	**Maximum Units***
eladocagene exuparvovec-tneq (Kebilidi®) intraputaminal infusion	AADC deficiency	Total recommended dose of 1.8 x 10^{11} vector genomes (vg), administered as four intraputaminal infusions in a single stereotactic neurosurgical procedure	C9399** J3490** J3590**	**1**

***Maximum units allowed for duration of approval**
****Non-specific assigned HCPCS codes, must submit requested product NDC**

Other revenue codes that may be applicable to this policy: 0891, 0892

References: all information referenced is from FDA package insert unless otherwise noted below.

1. 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;12(12):1-21.

Policy Implementation/Update Information: Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q4 annually.

April 2026: Coding change: Added the following applicable revenue codes associated with policy HCPCS code(s): 0891 (Special Processed Drugs – FDA Approved Cell Therapy) and 0892 (Special Processed Drugs – FDA Approved Gene Therapy). **Policy notification given 2/1/2026 for effective date 4/1/2026**.
January 2025: Original medical policy criteria issued.