CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Arkansas PASSE

DRUG NAME	Kebilidi (eladocagene exuparvovec-tneq)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Kebilidi, approved by the FDA in 2024, is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of adult and pediatric patients with aromatic L amino acid decarboxylase (AADC) deficiency. It has accelerated approval status based on change from baseline in gross motor milestone achievement at 48 weeks post-treatment compared to a natural history cohort.
AADC deficiency is a rare neurometabolic disorder with a severe combined deficiency of serotonin, dopamine, norepinephrine, and epinephrine. Key symptoms include hypotonia, movement disorders (oculogyric crisis, dystonia, and hypokinesia), developmental delay, and autonomic symptoms.
Kebilidi is administered as a single-dose intraputaminal infusion. It delivers a copy of the DDC gene which encodes the AADC enzyme, resulting in AADC enzyme expression and subsequent production of dopamine in the putamen.

Kebilidi (eladocagene exuparvovec) will be considered for coverage when the following criteria are met:

Aromatic L-amino acid decarboxylase (AADC) Deficiency

For **initial** authorization:
1. Member is at least 16 months of age; AND
2. Medication must be prescribed by a neurologist, neurosurgeon, neurometabolic specialist, or geneticist; AND
3. Member has a diagnosis of AADC deficiency confirmed by both of the following:
   a) Genetic test results that show biallelic mutations in the *DDC* gene, and
   b) Decreased AADC enzyme activity in plasma; AND
4. Member has a severe phenotype, defined as both of the following:
   a) No or very limited motor milestone achievement, and
   b) Persistent neurological symptoms despite standard of care therapies (i.e., selective dopamine agonists, MAO-inhibitors, and pyridoxine); AND
5. Documentation of skull maturity has been confirmed by neuroimaging (not required for adults).
6. **Dosage allowed/Quantity limit:** 1.8×10^{11} vg (0.32 mL total volume) delivered as four 0.08 mL (0.45×10^{11} vg) infusions (two sites per putamen-anterior and posterior).

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:
1. Kebilidi will not be approved for continued use. It is a one-time treatment.

CareSource considers Kebilidi (eladocagene exuparvovec) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
12/17/2024	New policy for Kebilidi created.

References:

1. Kebilidi [prescribing information]. PTC Therapeutics, Inc.; 2024.
2. Tai CH, Lee NC, Chien YH, et al. Long-term efficacy and safety of eladocagene exuparvovec in patients with AADC deficiency. *Mol Ther*. 2022;30(2):509-518. doi:10.1016/j.ymthe.2021.11.005
3. 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(1):12. Published 2017 Jan 18. doi:10.1186/s13023-016-0522-z
4. Roubertie A, Opladen T, Brennenstuhl H, et al. Gene therapy for aromatic L-amino acid decarboxylase deficiency: Requirements for safe application and knowledge-generating follow-up. *J Inherit Metab Dis*. 2024;47(3):463-475. doi:10.1002/jimd.12649
5. Pearson TS, Gilbert L, Opladen T, et al. AADC deficiency from infancy to adulthood: Symptoms and developmental outcome in an international cohort of 63 patients. *J Inherit Metab Dis*. 2020;43(5):1121-1130. doi:10.1002/jimd.12247

Effective date: 07/01/2025
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