Reference number(s)
6952-A

Specialty Guideline Management
Kebilidi

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Kebilidi	eladocagene exuparvovec-tneq

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1]

Kebilidi is indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

This indication is approved under accelerated approval based on change from baseline in gross motor milestone achievement at 48 weeks post-treatment. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Genetic test results confirming the AADC diagnosis.

- Medical records (e.g., chart notes and/or laboratory reports) documenting following:
    - Cerebrospinal fluid (CSF) results
    - Skull maturity (radiology report required)

Prescriber Specialties

This medication must be prescribed by or in consultation with a neurologist, geneticist, or physician specializing in the treatment of inherited metabolic diseases.

Exclusions

Coverage will not be provided for members with any of the following conditions:

- Anti-adeno-associated virus, serotype 2 (anti-AAV2) antibody titers greater than 1:1200
- Pyridoxine 5'-phosphate oxidase or tetrahydrobiopterin (BH4) deficiency
- Evidence of a clinically active infection

Coverage Criteria

Aromatic L-amino acid decarboxylase (AADC) deficiency[1]

Authorization of 3 months for one dose total may be granted for treatment of AADC when all of the following criteria are met:

- Member is 16 months through 10 years of age.
- Member's cerebrospinal fluid (CSF) has abnormal levels of neurotransmitter metabolites associated with AADC deficiency (i.e., reduced levels of 5-hydroxyindoleacetic acid [5-HIAA], homovanillic acid [HVA] and 3-methoxy-4-hydroxyphenylglycol [MHPG]; with normal CSF pterins including neopterin and biopterin; and increased CSF levels of L-Dopa, 3-O-methyldopa [3-OMD] and 5-OH tryptophan [5-HTP]).
- Member has decreased AADC activity in the plasma.
- Member has documented AADC deficiency due to biallelic variants in the DDC gene
- Member has persistent neurological defects (e.g., developmental delays, movement disorders [dystonia, hypokinesia, hypotonia, oculogyric crises], autonomic dysfunction [hyperhidrosis, hypersalivation, hypotension, hypoglycemia, ptosis], intellectual disability) despite standard medical therapy (i.e., dopamine agonists, monoamine oxidase inhibitor, pyridoxine or other forms of vitamin B6).
- Member is unable to ambulate independently with or without assistive devices.

- Skull maturity of member is sufficient for stereotaxis, namely development of three distinct skull layers (inner and outer cortical and middle cancellous), is documented on a formal radiology report.
- Brain MRI will be used for stereotactic planning and intraoperative navigation.
- Kebilidi will be administered in a medical center which specializes in stereotactic neurosurgery.
- Dose will not exceed 1.8×10^{11} vector genomes (vg).

References

1. Kebilidi [package insert]. Warren, NJ: PTC Therapeutics, Inc.; November 2024.