Reference number(s)
7025-A

Standard Medicare Part B 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 will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

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Chart notes, medical records, or lab results documenting all of the following:

- Genetic test results confirming the aromatic L-amino acid decarboxylase (AADC) diagnosis
- Skull maturity (radiology report)

Coverage Criteria

Aromatic L-amino Acid Decarboxylase (AADC) Deficiency[1]

Authorization of 3 months for a one-time administration may be granted for the treatment of AADC when all of the following criteria are met:

- Member is 16 months through 10 years of age.
- Member has genetically confirmed AADC deficiency due to biallelic variants in the DDC gene.
- Member has decreased AADC activity in the plasma.
- Skull maturity has been assessed with neuroimaging and is sufficient for stereotaxis, namely development of three distinct skull layers (inner and outer cortical and middle cancellous).
- Member does not have anti-adeno-associated virus, serotype 2 (anti-AAV2) antibody titers greater than 1:1200.
- Dose will not exceed 1.8×10^{11} vector genomes (vg).

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Kebilidi.
- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Kebilidi are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

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References

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

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