

Medical Policy Manual

Draft New Policy: Do Not Implement

Eladocogene exuparvovec-tneq (Kebilidi®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

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

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 SPECIALITIES

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-adenovirus-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

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COVERAGE CRITERIA

Aromatic L-amino Acid Decarboxylase (AADC) Deficiency

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).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

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

EFFECTIVE DATE

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**BlueCross BlueShield
of Tennessee**

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