

DRUG POLICY

Kebilidi® (eladocagene exuparvovec-tneq)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

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 clinical trial.

POLICY

Required Documentation

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

1. Genetic testing results documenting biallelic mutations in the DOPA decarboxylase (DDC) gene
2. Chart notes, medical records or laboratory values documenting reduced levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA) and 3-methoxy-4-hydroxyphenylglycol (MHPG) and high concentrations of 3-O-methyldopa (3-OMD), L-Dopa, and 5-OH tryptophan (5-HTP) in the cerebral spinal fluid (CSF)
3. Chart notes, medical records or laboratory values documenting reduced aromatic L-amino acid decarboxylase (AADC) activity in the plasma

Prescriber Specialties

The requested medication must be prescribed by or in consultation with one of the following:

1. Pediatric neurologist
2. Movement disorder specialist
3. Clinical geneticist specializing in the management of AADC deficiency

Criteria for Initial Approval

Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Authorization of a single dose per lifetime may be granted for AADC deficiency when all of the following criteria are met:

1. Patient has genetically confirmed AADC deficiency as defined by all of the following criteria:
 - A. Genetic testing showing biallelic mutations in the DOPA decarboxylase (DDC) gene
 - B. Reduced levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA) and 3-methoxy-4-hydroxyphenylglycol (MHPG) and high concentrations of 3-O-methyldopa (3-OMD), L-Dopa, and 5-OH tryptophan (5-HTP) in the cerebral spinal fluid (CSF)
 - C. Reduced aromatic L-amino acid decarboxylase (AADC) activity in the plasma
2. Patient is aged 16 months or older
3. Patient achieved skull maturity as assessed with neuroimaging
4. Patient does not have any significant structural brain abnormality
5. Patient has severe AADC deficiency at baseline as demonstrated by not meeting any gross motor milestones (i.e., inability to sit, stand, or ambulate independently)
6. Patient has the presence of one or more classical clinical characteristics associated with AADC deficiency (i.e., oculogyric crises, hypokinesia, hypotonia, developmental delays, ptosis, autonomic dysfunction)
7. Patient continues to experience neurological defects associated with AADC deficiency despite standard medical therapy (i.e., dopamine agonists, monoamine oxidase inhibitor, pyridoxine)
8. Patient does not have an anti-AAV2 neutralizing antibody titer over 1,200 folds or an enzyme-linked immunoassay optical density over 1
9. Patient does not have clinically active infection
10. Patient does not have a contraindication for imaging studies (i.e. computed tomography [CT] scan, positron emission tomography [PET] scan) or magnetic resonance imaging (MRI)
11. Administration will occur in a medical center that specializes in stereotactic neurosurgery
12. The patient does not have history of receiving prior treatment with any gene therapy for any indication in their lifetime

Continuation of Therapy

Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Repeat treatment of Kebilidi for any indication is considered investigational, as the safety and efficacy beyond one dose has not been studied. Approval is limited to one treatment course per lifetime.

Other

Kebilidi (eladocogene exuparvovec-tneq) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Dosing and Administration

The recommended dose is 1.8×10^{11} vector genomes given in 4 intraputaminial infusions of 0.45×10^{11} vector genomes per dose in single stereotactic neurosurgical procedure.

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits Apply

Kebilidi approvals will be limited to one treatment per lifetime

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- C9399 – Unclassified drugs or biologicals (when specified as Kebilidi [eladocagene exuparvovec])
- J3590 – Unclassified biologics (when specified as Kebilidi [eladocagene exuparvovec])
- J3490 – Unclassified drugs (when specified as Kebilidi [eladocagene exuparvovec])

REFERENCES

Kebilidi [prescribing information]. Warren, NJ: PTC Therapeutics, Inc.; November 2024.

Clinicaltrials.gov. An open-label trial to address the safety of the SmartFlow MR-compatible ventricular cannula for administering eladocagene exuparvovec to pediatric subjects (NCT04903288). Available at: <https://clinicaltrials.gov/study/NCT04903288>. Accessed on March 18, 2025.

Chien YH, Lee NC, Tseng SH, et al. AGIL-AADC gene therapy results in sustained improvements in motor and developmental milestones over 5 years in children with AADC deficiency. *Neuro*. 2019 April 9; 92 (Suppl 15): 4.6 – 058.

Hwu PWL, Chein YH, Lee NC, et al. Safety and improved efficacy outcomes in children AADC deficiency treated with eladocagene exuparvovec gene therapy: results from three clinical trials. *Neuro*. 2020 April 14; 94 (Suppl 15): 4688.

Chien YH, Lee NC, Tseng SH, et al. Efficacy and safety of AAV2 gene therapy in children with aromatic L-amino acid decarboxylase deficiency: an open-label, phase 1/2 trial. *Lancet Child Adolesc Health*. 2017 Dec 1; 1 (4): 265-273.

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 Dec; 12 (1): 12 – 33.

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POLICY HISTORY

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