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Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	February 21, 2025
Subject:	Kebilidi	**Page:**	1 of 4

Last Review Date: March 7, 2025

Kebilidi

Description

Kebilidi (eladocagene exuparvovec-tneq)

Background
Aromatic L-amino acid decarboxylase (AADC) deficiency is a disorder involving complex symptoms, including motor, behavioral, cognitive, and autonomic findings. Symptom onset is in the early infancy, typically within the first six months of life. The AADC enzyme catalyzes the last step in the biosynthesis of dopamine and serotonin. Dopamine itself is a precursor for the synthesis of epinephrine and norepinephrine. Therefore, the clinical features of AADC deficiency are cause by a severe combined deficiency of dopamine, serotonin, epinephrine, and norepinephrine (1).

Kebilidi (eladocagene exuparvovec-tneq) is a recombinant adeno-associated virus serotype 2 (rAAV2) based gene therapy designed to deliver a copy of the DOPA decarboxylase (DDC) gene which encodes the AADC enzyme. Intraputaminal infusion of Kebilidi results in AADC enzyme expression and subsequent production of dopamine in the putamen (2).

Regulatory Status
FDA-approved indication: Kebilidi 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 (2).

Kebilidi is contraindicated in patients who have not achieved skull maturity assessed by neuroimaging. Skull maturity is needed for stereotactic neurosurgical administration of Kebilidi (2).

Kebilidi has been associated with procedural complications and dyskinesia. Monitor patients for procedural complications for neurosurgery, including events of respiratory and cardiac arrest after administration of Kebilidi. Dyskinesia has been reported after administration of Kebilidi. Monitor for signs and symptoms of dyskinesia after treatment and consider the use of dopamine antagonists to control symptoms (2).

The diagnosis of AADC deficiency is established in patients with the following diagnostic test results: biallelic variants in the DDC gene, reduced levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA), and 3-mthoxy-4-hydroxyphenylglycol (MHPG) in the cerebral spinal fluid (CSF), high concentrations of 3-O-methyldopa (3-OMD), levodopa, and 5-hydroxytryptophan (5-HTP) in the CSF, and reduced AADC activity in the plasma (1).

The safety and effectiveness of Kebilidi in pediatric patients less than 16 months of age have not been established (2).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Kebilidi may be considered **medically necessary** if the conditions indicated below are met.

Kebilidi may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 16 months of age or older

Diagnosis

Patient must have the following:

Aromatic L-amino acid decarboxylase (AADC) deficiency

AND ALL of the following:

1. The diagnosis of AADC deficiency must be confirmed by **ALL** of the following:
    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) in the cerebral spinal fluid (CSF)
    c. High concentrations of 3-O-methyldopa (3-OMD), levodopa, and 5-hydroxytryptophan (5-HTP) in the cerebral spinal fluid
    d. Reduced aromatic L-amino acid decarboxylase (AADC) activity in the plasma
2. Patient has achieved skull maturity assessed by neuroimaging
3. Prescribed by or in consultation with a pediatric neurologist

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity One vial (only one PA approval for one treatment per lifetime)

Prior – Approval *Renewal* Limits

None

Rationale

Summary

Kebilidi is an AVV vector-based gene therapy indicated for the treatment of patients with AADC deficiency. Kebilidi is contraindicated in patients who have not achieved skull maturity assessed by neuroimaging. The safety and effectiveness of Kebilidi in pediatric patients less than 16 months of age have not been established (2).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Kebilidi while maintaining optimal therapeutic outcomes.

References

1. Blau N, Pearson TS, Kurian MA, et al. Aromatic L-Amino Acid Decarboxylase Deficiency. *GeneReviews*. 2023. https://www.ncbi.nlm.nih.gov/books/NBK595821/. Accessed January 23, 2025.
2. Kebilidi [package insert]. Warren, NJ: PTC Therapeutics, Inc.; November 2024.

Policy History

Date	Reason
February 2025	Addition to PA
March 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.