Kebilidi® (Eladocagene Exuparvovec-Tneq)

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➲ Instructions for Use

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Community Plan Policy

- Kebilidi® (Eladocagene Exuparvovec-Tneq)

Application

UnitedHealthcare Commercial

This Medical Policy applies to UnitedHealthcare Commercial benefit plans.

UnitedHealthcare Individual Exchange

This Medical Policy applies to Individual Exchange benefit plans.

Coverage Rationale

➲ See Benefit Considerations

Kebilidi is proven and medically necessary for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency in patients who meet all of the following criteria:

- Submission of medical records documenting **all** of the following:
  - Genetically confirmed AADC deficiency due to biallelic mutations in the dopa decarboxylase (*DDC*) gene; **and**
  - Patient has one or more of the following typical clinical characteristics associated with AADC deficiency (e.g., hypotonia, oculogyric crises, dystonia, hypokinesia, autonomic dysfunction, developmental delay); **and**
  - Decreased AADC enzyme activity in plasma per current laboratory standards

  and
- Patient has achieved skull maturity, as confirmed by neuroimaging, necessary for stereotactic neurosurgical administration of Kebilidi; **and**
- Patient has persistent symptoms of AADC deficiency (e.g., hypotonia, oculogyric crises, dystonia, hypokinesia, autonomic dysfunction, developmental delay) despite use of standard medical therapy (e.g., dopamine agonists, monoamine oxidase inhibitors, pyridoxine, other forms of vitamin B6); **and**
- Patient is unable to ambulate independently with or without an assistive device such as cane or walker; **and**
- Patient does not have an anti-adeno-associated virus, serotype 2 (anti-AAV2) antibody titer higher than 1:1200 or > 1 optical density value by enzyme-linked immunosorbent assay (ELISA); **and**
- Prescribed by a neurologist or neurosurgeon; **and**
- Patient has not previously received treatment with Kebilidi or other gene therapy for the treatment of AADC deficiency in their lifetime; **and**
- Dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
- Authorization will be issued for no more than one treatment per lifetime and for no longer than 60 days from approval

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Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other policies and guidelines may apply.

HCPCS Code	Description
C9399	Unclassified drugs or biologicals
J3590	Unclassified biologics

Diagnosis Code	Description
E70.81	Aromatic L-amino acid decarboxylase (AADC) deficiency

Background

Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare genetic neurological disorder arising from biallelic pathological variants in the dopa decarboxylase (DDC) gene that encodes for the AADC enzyme. Deficiency of the AADC enzyme leads to an inability to synthesize dopamine and serotonin from their precursors, L-3,4- dihydroxyphenylalanine (L-DOPA) and 5-hydroxytryptophan (5- HTP). Without neuronal dopamine, patients suffer from movement disorders including hypokinesia, dystonia, and oculogyric crisis that result in motor dysfunction, along with behavioral problems, autonomic dysfunction, and developmental delay.

Kebilidi (eladocagene exuparvovec-tneq) is a one-time gene replacement therapy that delivers a copy of the DDC gene via a recombinant adeno-associated virus serotype 2 (AAV2) to cells within the putamen resulting in AADC enzyme expression and subsequent production of dopamine.

Benefit Considerations

Some Certificates of Coverage allow for coverage of experimental/investigational/unproven treatments for life-threatening illnesses when certain conditions are met. The member specific benefit plan document must be consulted to make coverage decisions for this service. Some states mandate benefit coverage for off-label use of medications for some diagnoses or under some circumstances when certain conditions are met. Where such mandates apply, they supersede language in the member specific benefit plan document or in the medical or drug policy.

Clinical Evidence

Proven

The efficacy of Kebilidi was evaluated in one 48-week, Phase 2, open-label, single arm study (NCT04903288). The trial consisted of a trial phase (8 weeks), an extension phase (to 48 weeks), and an ongoing long-term extension phase (to 260 weeks). All patients (n = 13) received a total dose of 1.8×10^{11} vector genome given as 4 intraputaminal infusions in a single stereotactic neurosurgical procedure. Select outcomes were compared to an external untreated natural history cohort of 44 pediatric patients with severe AADC deficiency with ≥ 1 motor milestone assessment after 2 years of age. Included patients were ages 1 to < 18 years old with genetically confirmed, severe AADC deficiency, decreased AADC enzyme activity in the plasma, and skull maturity appropriate for the procedure. Patients were also required to have persistent neurological defects secondary to AADC deficiency despite standard medical therapy and be unable to ambulate independently. At baseline, the median age was 2.8 years (range, 1.3 to 10.8 years), 7 patients (54%) were female, and 10 patients (77%) were Asian. Only 1 patient was ≥ 6 years of age at the time of treatment. Nearly all were considered to have a severe phenotype with no motor milestone achievement, and 1 other had a “variant” severe phenotype with the ability to sit with assistance but with lack of head control. The primary enpoint was the change from baseline in the CSF homovanillic acid (HVA) level, a metabolite of dopamine, at Week 8. In all 13 patients, CSF HVA increased from baseline, but there was also substantial intrapatient and intervariability in CSF HVA levels. A secondary endpoint, gross motor milestone achievement evaluated at week 48, was assessed using the Peabody Developmental Motor Scale, Second Edition (PDMS-2) in 12 of the 13 patients treated (one patient dropped out of the study prior to week 48). Eight (67%) of the 12 treated patients achieved a new gross motor milestone at week 48: 3 patients achieved full head control, 2 patients achieved sitting with or without assistance, 2 patients achieved walking backwards and the patient

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with the “variant” severe phenotype was able to sit unassisted. The two patients who achieved walking backwards at week 48 were treated before 2 years of age. The four patients who were unable to achieve new gross motor milestones at week 48 were treated between the ages of 2.8 and 10.8 years. In comparison, 0 of the 44 untreated patients with the severe phenotype had documented motor milestone achievement at last assessment at a median age of 7.2 years (range 1.6 to 21 years).

Professional Societies

International Working Group on Neurotransmitter Related Disorders

In 2017, representatives of the International Working Group on Neurotransmitter Related Disorders (iNTD) and patient representatives evaluated all available evidence for diagnosis and treatment of AADCD and made recommendations using SIGN and GRADE methodology. In the face of limited definitive evidence, practical recommendations were constructed on clinical diagnosis, laboratory diagnosis, imaging and electroencephalograpy, medical treatments and non-medical treatments. The core recommendations for diagnosis and medical treatment of AADC deficiency were as follows (strong recommendation):

- There are three core diagnostic tools for identifying AADCD:
  - Low CSF levels of 5-HIAA, HVA and MHPG, with normal CSF pterins, and increased CSF levels of LDopa, 3-OMD and 5-HTP
  - Genetic diagnosis showing compound heterozygous or homozygous disease causing variants in the DDC gene
  - Decreased AADC enzyme activity in plasma

To diagnose AADCD, genetic testing should be performed and at least two out of three core diagnostic tests should be positive. If local resources allow, we recommend performing all three key diagnostic tests in patients with this rare disorder.

- The core recommendations for treatment of AADCD are:
  - First line treatment with selective dopamine agonists, MAO-inhibitors, and pyridoxine
  - Additional symptomatic treatment agents with anticholinergic agents, melatonin, benzodiazepines, and alpha-adrenoreceptor blockers
  - In general, multiple drug classes will be needed
  - Overall treatment principles to adhere to are stepwise approach, start low and go slow when increasing dosages, and discontinue/ gradually withdraw medication that is not helpful

Involvement of a multidisciplinary team, including physiatrist and paramedical therapy services, is essential in the care for AADCD patients, and psychological support should be offered to caregivers, siblings, and patients. Patients and caregivers should be informed about the AADCD parental organizations. AADCD patients should be seen at least yearly by a (child) neurologist experience in movement disorders or neurometabolic disease, ideally in a multidisciplinary setting, facilitating a shared-care approach.

U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

Kebilidi (eladocagene exuparvovec-tneq) is an adeno-associated virus (AVV) vector-based gene therapy indicated for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency.

This indication is approved under accelerated approval based on change from 16 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.

References

1. Kebilidi® [package insert]. Warren, NJ: PTC Therapeutics, Inc.; November 2024.
2. An open-label trial to address the safety of the SmartFLow MR-Compatible Ventricular Cannula for administering eladocagene exuparvovec to pediatric subjects. ClinicalTrials.gov identifier: NCT04903288. Updated March 6, 2025. Accessed March 20, 2025. https://www.clinicaltrials.gov/ct2/show/NCT04903288.
3. Wassenberg T, Molero-Luis M, Jeltsch K, et a. Consensus guideline for the diagnosis and treatment of aromatic l-amino acid decarboxylase (AADC) deficiency. *Orphanet J Rare Dis*. 2017;12(1):12.

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Policy History/Revision Information

Date	Summary of Changes

08/01/2026	**Template Update**
	• Transferred content to shared policy template that applies to both UnitedHealthcare Commercial and Individual Exchange benefit plans
	• Added *Application* section to indicate this policy applies to:
	  ○ UnitedHealthcare Commercial benefit plans
	  ○ Individual Exchange benefit plans
	Coverage Rationale
	• Revised coverage criteria; replaced criterion requiring "the patient is unable to ambulate independently" with "the patient is unable to ambulate independently *with or without an assistive device such as cane or walker*"
	Supporting Information
	• Archived previous policy versions 2025D0136A and IEXD0136.01

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting UnitedHealthcare standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, check the member specific benefit plan document and any applicable federal or state mandates. UnitedHealthcare reserves the right to modify its policies and guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

This Medical Benefit Drug Policy may also be applied to Medicare Advantage plans in certain instances. In the absence of a Medicare National Coverage Determination (NCD), Local Coverage Determination (LCD), or other Medicare coverage guidance, CMS allows a Medicare Advantage Organization (MAO) to create its own coverage determinations, using objective evidence-based rationale relying on authoritative evidence (Medicare IOM Pub. No. 100-16, Ch. 4, §90.5).

UnitedHealthcare may also use tools developed by third parties, such as the InterQual® criteria, to assist us in administering health benefits. UnitedHealthcare Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.

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