

## Kebilidi (eladocagene exuparvovec-tneq)

Effective: October 1, 2025

### Prior Authorization Required

If **REQUIRED**, submit supporting clinical documentation pertinent to service request.

Yes ☒ No ☐

### Applies to:

- ☒ CarePartners of Connecticut Medicare Advantage HMO plans, Fax 617-673-0956
- ☒ CarePartners of Connecticut Medicare Advantage PPO plans, Fax 617-673-0956

**Note:** While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

## Overview

Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare inherited (autosomal recessive) disorder characterized by defective synthesis of dopamine and serotonin. It is caused by mutations in the dopa decarboxylase (DDC) gene encoding the AADC enzyme, resulting in marked dopamine loss that severely impedes normal motor development (head control, sitting, standing, and walking). The onset of the condition is usually in early infancy. Symptoms of AADC include developmental delay, cognitive issues (intellectual disability), motor issues (hypokinesia, dystonia, oculogyric crises), and autonomic dysfunction (hypoglycemia and temperature instability). Respiratory infections and pneumonia are primary causes of morbidity in patients with AADC deficiency. AADC deficiency can be life-threatening and people with this condition are at a significant risk of death, especially in the first decade of life due to the severe complications it causes. Incidence of AADC is estimated to be 1 to 3 per 1,000,000 live births. The Peabody Developmental Motor Scale, Second Edition (PDMS-2) is a tool used to evaluate patient response. The overall PDMS-2 involves six subtests that evaluate motor abilities that develop early in life (e.g., grasping, visual-motor integration, reflexes, stationary, locomotion, and object manipulation).

### Food and Drug Administration (FDA) Approved Indications:

- 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.

For single-dose intraputamen infusion only. Skull maturity is needed for stereotactic neurosurgical administration of Kebilidi. Brain imaging for stereotactic planning and intraoperative navigation 24 should be done prior to the procedure. The total recommended dose is 1.8 x 10<sup>11</sup> vector genomes (0.32 mL) which is administered by four intraputamen infusions in a single stereotactic neurosurgical procedure. Kebilidi is intended to be given with an infusion pump that is able to permit infusion at a rate of 0.003 mL/minute. Two infusions are delivered in the anterior putamen and two in the posterior putamen. At each target point, the duration of the infusion is 27 minutes. Kebilidi should be administered only using an FDA-authorized cannula for intraparenchymal infusion.

Monitor patients for procedural complications for neurosurgery, including events of respiratory and cardiac arrest after administration of KEBILIDI. Monitor patients for dyskinesia after treatment with KEBILIDI. The use of dopamine antagonists can be used to control dyskinesia symptoms.

The Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) and MassHealth for coverage determinations for its Dual Product Eligible plan members and CMS for its Medicare Advantage plan members. CMS National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs) and documentation included in the Medicare manuals and MassHealth Medical Necessity Determinations are the basis for coverage determinations. When CMS and MassHealth do not provide guidance, the Plan's internally developed medical necessity guidelines are used. CMS coverage guidelines are not established for this service. Point32Health covers Kebilidi in accordance with MassHealth coverage criteria.

For the therapy Kebilidi, evidence is sufficient for coverage. Kebilidi received FDA approval in November 2024 supported by the results of the PTC-AADC-GT-002 phase 2, open-label, single arm trial. The main outcome measure was gross motor milestone achievement evaluated at week 48 and assessed using the Peabody Developmental Motor Scale, Second Edition (PDMS-2). Eight (67%) of the 12 treated patients achieved a new gross motor milestone at week 48. In comparison, none of the 43 untreated

patients with severe phenotype had documented motor milestone achievement at last assessment at a median age of 7.2 years. Kebilidi provides an additional treatment option for a very specific patient population with AADC deficiency. The use of this criteria in the utilization management process will ensure access to evidence based clinically appropriate care. See References section below for all evidence accessed in the development of these criteria.

### Clinical Guideline Coverage Criteria

The Plan may cover Kebilidi when all the following clinical criteria is met:

- 1. Documentation of diagnosis of aromatic L-amino acid decarboxylase (AADC) deficiency; **and**
- 2. Copy of genetic test confirming biallelic mutation of the DDC gene to support a diagnosis of AADC deficiency; **and**
- 3. Laboratory test results of **ONE** of the following:
  - a. Decreased AADC enzyme activity in plasma.
  - b. Cerebrospinal fluid showing **BOTH** of the following:
    - i. Decreased levels of 5-HIAA, HV, and MHPG
    - ii. Increased levels of 3-OMD, L-Dopa, and 5-HTP
- 4. The Member is ≥16 months of age; **and**
- 5. The Prescriber is a neurologist or consult notes from a neurologist are provided); **and**
- 6. The Member has achieved skull maturity required for stereotactic surgical administration (provider attestation may be accepted); **and**
- 7. Medical records documenting **BOTH** of the following:
  - a. The Member is unable to ambulate independently.
  - b. The Member is experiencing neurological defects despite treatment with a dopamine agonist, monoamine oxidase inhibitor and/or vitamin B6.
- 8. Appropriate dosing and treatment dates; **and**
- 9. The Member has not received any prior gene therapy for AADC deficiency.

**Note:** The Member is limited to a maximum of one treatment course with this regimen.

### Codes

The following code(s) require prior authorization:

**Table 1: CPT/HCPCS Codes**

HCPCS Codes	Description
None	

### References:

- 1. Kebilidi (eladocagene exuparvovec-tneq) [package insert]. Warren, NJ: PTC Therapeutics. November 2024
- 2. Kebilidi. Fda.gov. November 13, 2024 Summary Basis for Regulatory Action – KEBILIDI. Available at: <https://www.fda.gov/media/184353/download?attachment>
- 3. Clinicaltrials.gov. A Study of SmartFlow Magnetic Resonance (MR) Compatible Ventricular Cannula for Administering Eladocagene Exuparvovec to Pediatric Participants (NCT04903288). Available at: <https://clinicaltrials.gov/study/NCT04903288>. Accessed on January 22, 2025.
- 4. Clinicaltrials.gov. A Phase I/II Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC (AADC). (NCT01395641). Available at: <https://clinicaltrials.gov/study/NCT01395641>. Accessed on January 24, 2025.
- 5. Clinicaltrials.gov. A Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC - An Expansion. (NCT02926066). Available at: <https://clinicaltrials.gov/study/NCT02926066>. Accessed on January 24, 2025.
- 6. 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;12(1):12. Published 2017 Jan 18. doi:10.1186/s13023-016-0522-z
- 7. IPD Analytics New Drug Review; Kebilidi (Eladocagene exuparvovec-tneq). Available at <https://www.ipdanalytics.com/> [subscription required].
- 8. Aromatic L-Amino Acid Decarboxylase Deficiency. rarediseases.org. <https://rarediseases.org/rare-diseases/aromatic-l-amino-acid-decarboxylase-deficiency/>. Published 2024. Last updated November 14, 2024.

9. Simons CL, Hwu WL, Zhang R, Simons MJHG, Bergkvist M, Bennison C. Long-Term Outcomes of Eladocagene Exuparvovec Compared with Standard of Care in Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency: A Modelling Study. *Adv Ther.* 2023;40(12):5399-5414. doi:10.1007/s12325-023-02689-6
10. Tai CH, Lee NC, Chien YH, et al. Long-term efficacy and safety of eladocagene exuparvovec in patients with AADC deficiency. *Mol Ther.* 2022;30(2):509-518. doi:10.1016/j.ymthe.2021.11.005
11. Valentini NC, Zanella LW. Peabody Developmental Motor Scales-2: The Use of Rasch Analysis to Examine the Model Unidimensionality, Motor Function, and Item Difficulty. *Front Pediatr.* 2022;10:852732. Published 2022 Apr 20. doi:10.3389/fped.2022.852732
12. MassHealth Drug List - health and human services. Table 65: Enzyme and Metabolic Disorder Therapies. July 1, 2025. Accessed July 8, 2025. <https://mhdL.pharmacy.services.conduent.com/MHDL/pubtheradetail.do?id=2904&drugId=9298>

---

## Approval And Revision History

July 16, 2025: Reviewed by the Medical Policy Approval Committee (MPAC). Effective date October 1, 2025.

---

## Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.