Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines

Medical Benefit Drugs

Kebilidi (eladocagene exuparvovec-tneq)

Effective: July 1, 2026

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☒ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☐ Tufts Health One Care-- A dual-eligible product; Fax 617-673-0956

Senior Products

☐ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☐ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☐ Tufts Medicare Preferred PPO, (a Medicare Advantage product); 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 intraputaminal 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 1011 vector genomes (0.32 mL) which is administered by four intraputaminal 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.

Clinical Guideline Coverage Criteria

The Plan may cover a **one-time only dose** of Kebilidi (eladocagene exuparvovec-tneq) when **all** the following clinical criteria is met:

1. The Member is ≥16 months of age; and
2. Documentation of aromatic L-amino acid decarboxylase (AADC) deficiency with the diagnosis confirmed based on **all** the following:
    a. Genetic testing showing biallelic variants in the DOPA decarboxylase (DDC) gene; and
    b. Decreased aromatic L-amino acid decarboxylase (AADC) enzyme activity in plasma; and
    c. Lumbar puncture to determine cerebrospinal fluid (CSF) for abnormal levels of neurotransmitter metabolites associated with AADC deficiency.
3. The Member has documented skull maturity as assessed by neuroimaging (closed anterior fontanelle); and
4. The Member presents with an inability to sit, stand, or walk independently; and
5. The Member has persistent neurological deficits of AADC deficiency such as hypotonia, dystonia, developmental delay, and oculogyric crises despite use of at least one standard medication therapy (monoamine oxidase inhibitor, dopamine agonists, pyridoxine, or other types of vitamin B6); and
6. Brain MRI has been conducted for stereotactic planning and intraoperative navigation; and
7. The Prescribing physician is a neurologist or neurosurgeon; and
8. Kebilidi will be administered in a medical center which specializes in stereotactic neurosurgery; and
9. Dosing of Kebilidi is in accordance with the FDA dosing recommendations and will not exceed 1.8×10^{11} vector genomes which is administered by four intraputaminal infusions (0.08 mL each at a dose of 0.45×10^{11} vector genomes) in a single stereotactic neurosurgical procedure.

NOTE: The Plan considers additional indications not listed above as experimental, unproven, or investigational and therefore not medically necessary given the lack of scientific evidence to support that the treatment is effective in improving health outcomes.

Limitations

The plan will not cover Kebilidi (eladocagene exuparvovec-tneq) under any of the following conditions:

- The Member has pyridoxine 5'-phosphate oxidase or tetrahydrobiopterin (BH4) deficiency
- The Member has anti-adeno-associated virus, serotype 2 (anti-AAV2) antibody titers greater than 1:1200

Codes

The following code(s) require prior authorization:

Table 1: CPT/HCPCS Codes

Codes	Description
None	

References:

1. Kebilidi (eladocagene exuparvovec-tneq) [package insert]. Warren, NJ: PTC Therapeutics. November 2024
2. 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.
3. 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.
4. 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.
5. Himmelreich N, Montioli R, Bertoldi M, et al. Aromatic amino acid decarboxylase deficiency: Molecular and metabolic basis and therapeutic outlook [published correction appears in Mol Genet Metab. 2021 Sep-Oct;134(1-2):216. doi: 10.1016/j.ymgme.2021.06.010.]. Mol Genet Metab. 2019;127(1):12-22. doi:10.1016/j.ymgme.2019.03.009
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. Eladocagene Exuparvovec-tneq (Kebilidi; PTC Therapeutics) for Aromatic L-amino Acid Decarboxylase Deficiency. Hayesinc.com/login [**via subscription only]**. Published November 18, 2024. Accessed January 15, 2025.
8. IPD Analytics New Drug Review; Kebilidi (Eladocagene exuparvovec-tneq). Available at https://www.ipdanalytics.com/ [**subscription required].**
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10. 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
11. Package Insert- Kebilidi
12. 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
13. 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
14. Kebilidi. Fda.gov. November 13, 2024 Summary Basis for Regulatory Action – KEBILIDI. Available at: https://www.fda.gov/media/184353/download?attachment

Approval And Revision History

February 18, 2026: Reviewed by the Medical Policy Approval Committee (MPAC), effective April 1, 2026.

Subsequent endorsement date(s) and changes made:

- May 20, 2026: Reviewed by MPAC, renewed without changes effective July 1, 2026.

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.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

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.