Pharmacy Management Drug Policy

SUBJECT: Kebilidi (eladocagene exuparvovec-tneq)
POLICY NUMBER: PHARMACY-128
EFFECTIVE DATE: 02/20/2025
LAST REVIEW DATE: 11/19/2025

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Kebilidi is a gene therapy product indicated for adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency that uses a modified adeno-associated virus serotype 2 (AAV2) to deliver a functional version of the DDC gene, which encodes the AADC enzyme, directly into the brain, resulting in AADC enzyme expression and subsequent production of dopamine in the putamen.

AACD deficiency is a rare, fatal genetic disorder that often presents symptoms in the first few months of life. It results in low energy, involuntary movements, poor sleep, weak or stiff muscles and the failure to reach typical achievements, such as learning to sit up, talk and walk. Many patients have oculogyric crises, which are typified by uncontrollable eye, head and neck movements, intense irritability, muscle spasms, pain and seizures. Oculogyric crises are painful and tend to occur every few days and can last for several hours. AADC deficiency also causes dysfunction of the autonomic nervous system causing many patients to experience low blood sugar, low blood pressure or excessive sweating.

Kebilidi is administered as four 0.8 mL (0.45 × 1011 vg) intraputaminal infusions in a single stereotactic neurosurgical procedure. Kebilidi was granted accelerated approval by the FDA based on safety and efficacy results from the Phase 2 PTC-AADC-GT-002 (NCT04903288) trial (referred to as Study 1 in the Kebilidi Prescribing Information), an ongoing, open-label, global study that enrolled 13 pediatric patients with genetically confirmed, severe AADC deficiency who had achieved skull maturity assessed with neuroimaging. Patients were compared to an external untreated natural history cohort of 43 pediatric patients with severe AADC deficiency who had at least one motor milestone assessment after 2 years of age. Continued approval may be contingent upon verification and description of clinical benefit in a confirmatory trial.

The main efficacy outcome measure, gross motor milestone achievement evaluated at Week 48, was assessed using the Peabody Developmental Motor Scale, Second Edition (PDMS-2).
The main efficacy outcome was evaluated in 12 of the 13 patients treated in the study (one patient dropped out of the study prior to Week 48). Eight (67%) patients achieved a new gross motor milestone at Week 48: three patients achieved full head control, two patients achieved sitting with or without assistance, two patients achieved walking backward, and the patient with the "variant"

severe phenotype was able to sit unassisted. The two patients who achieved walking backward 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 2.8 and 10.8 years of age. In comparison, none of the 43 untreated control patients with the severe phenotype had documented motor milestone achievement at last assessment at a median age of 7.2 years (range: 2–19 years).

From a safety perspective, the median duration of follow-up was 72 weeks (range: 23–109 weeks). All reports of dyskinesia, the most common adverse reaction (AR), were reported within 3 months of Kebilidi administration, with two events requiring hospitalization. Though two reports of dyskinesia required hospitalization, most cases involved non-severe, involuntary movements of face, arm, leg, or entire body.

POLICY:

Kebilidi (eladocagene exuparvovec-tneq)
1. Must be prescribed by a neurosurgeon trained in stereotactic neurosurgery 2. Must have a diagnosis of AADC deficiency confirmed by genetic testing identifying biallelic mutations in the DDC gene 3. Patient must be unable to ambulate independently (with or without assistive device) 4. Patient does not have: a) Significant brain structure abnormality b) Anti-AAV2 neutralizing antibody titer >1:1200 or ELISA OD >1 c) Pyridoxine 5'-phosphate oxidase or BH4 deficiency 5. Kebilidi is indicated for one-time single-dose intraputaminal use only and therefore will not be authorized for retreatment. Retreatment will be considered Experimental/Investigational when any FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered 6. The recommended dose of Kebilidi is four 0.8 mL (0.45 × 1011 vg) intraputaminal infusions in a single stereotactic neurosurgical procedure a) Please refer to Kebilidi FDA-approve prescribing information for complete dosage and administration instructions 7. Kebilidi (eladocagene exuparvovec-tneq) is considered investigational when the above criteria are not met. 8. Kebilidi (eladocagene exuparvovec-tneq is considered investigational for all other indications. 9. Approval will be provided for 6 months to allow sufficient time for administration.

POLICY GUIDELINES:

1. Prior-authorization is contract dependent.
2. Clinical documentation must be submitted for each request (initial and recertification) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments/treatment history, diagnostic testing, laboratory test results, genetic testing/biomarker results, and imaging.
    - Continued approval at time of recertification will require documentation that the drug is providing ongoing benefit to the patient in terms of improvement or stability in disease state or condition. Such documentation may include progress notes, imaging or laboratory findings, and other objective or subjective measures of benefit which support that continued use of the requested product is medically necessary. Also, ongoing use of the requested product must continue to reflect the current policy's preferred formulary.
3. All non-FDA approved indications for Hemgenix will be evaluated using off label policy criteria.

4. Dose and frequency should be in accordance with the FDA label or recognized compendia (for off-label uses). When services are performed in excess of established parameters, they may be subject to review for medical necessity.
5. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
6. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. Indications that have not been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan (which may include review per the Health Plan's Off-Label Use of FDA Approved Drugs policy). Step therapy requirements may be imposed in addition to LCD/NCD requirements.
7. Not all contracts cover all Medical Infusible drugs. Refer to specific contract/benefit plan language for exclusions of Injectable Medications.
8. All requests will be reviewed to ensure they are being used for an appropriate indication and may be subject to an off-label review in accordance with our Off-Label Use of FDA Approved Drugs Policy (Pharmacy-32).
9. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
10. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.
CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:
Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).

HCPCS:

UPDATES:

Date	Revision
11/19/2025	Revised
10/01/2025	Revised
05/08/2025	Reviewed / P&T Committee Approval
04/14/2025	Revised & Implemented

REFERENCES:

1. Kebilidi™. Package insert. PTC Therapeutics;2024.