Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 344.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Kebilidi (eladocagene exuparvovec-tneq)

Applicable line of business:

Commercial	X	Medicaid	X
Medicare	X	ACA	X
CHIP			

I. Policy:
Kebilidi (eladocagene exuparvovec-tneq)

II. Purpose/Objective:
To provide a policy of coverage regarding Kebilidi (eladocagene exuparvovec-tneq)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than the department requiring/authoring the policy.
3. Devised – the date the policy was implemented.
4. Revised – the date of every revision to the policy, including typographical and grammatical changes.
5. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:
a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

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

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Kebilidi (eladocagene exuparvovec-tneq)will be considered medically necessary for the commercial, exchange, Medicare, and Medicaid lines of business when ALL of the following criteria are met:

- Medical record documentation of a diagnosis of aromatic L-amino acid decarboxylase (AADC) deficiency, as confirmed by decreased AADC enzyme activity in the plasma **AND**
- Medical record documentation of an AADC deficiency due to biallelic mutations in the Dopa Decarboxylase (DDC) gene **AND**
- Medical record documentation of persistent neurological defects* despite standard medical therapy (e.g. dopamine agonists, monoamine oxidase inhibitor, pyridoxine, or other forms of vitamin B6) **AND**
- Medical record documentation that member is unable to ambulate independently, with or without assistive device **AND**
- Medical record documentation of age greater than or equal to 16 months **AND**
- Medical record documentation that the patient has anti-adeno-associated virus serotype 2 (anti-AAV2) antibody titers ≤1:1200 **AND**
- Medical record documentation of achievement of skull maturity as assessed by neuroimaging **AND**
- Medical record documentation of completion of, or a plan to complete, brain imaging specifically for stereotactic planning and intraoperative navigation **AND**
- Medical record documentation that Kebilidi is prescribed by a neurologist, pediatric neurologist, geneticist, or physician specializing in the treatment of inherited metabolic diseases **AND**
- Medical record documentation of a prescribed dose that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature **AND**
- Medical record documentation that the patient has never received Kebilidi treatment in their lifetime **AND**
- Medical record documentation that the member has not received any previous gene therapy for any disease

*Per NORD, signs and symptoms of AADC deficiency include movement disorders (hypotonia, oculogyric crises, hypokinesia, hypertonia, dystonia, athetosis, chorea, tremors) and/or autonomic disorders (excessive sweating, hypersalivation, ptosis, nasal congestion, temperature instability, hypotension, hypoglycemia, seizures, irritability, excessive crying, insomnia, hypersomnia, hyporeflexia, hyperreflexia).

Note: The dosing of Kebilidi is a single total dose of 1.8×10^{11} vg (0.32 mL), given as four (4) intraputaminal infusions at a dose of 0.45×10^{11} vg (0.08 mL) per infusion.

AUTHORIZATION DURATION: One (1) time approval per lifetime (auth duration: 2 months). Requests for authorizations exceeding these limits will require the following medical record documentation of peer-reviewed literature citing well-designed clinical trials to indicate that the member's healthcare outcome will be improved by dosing beyond the FDA-approved treatment duration.

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

LINE OF BUSINESS:
Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Kebilidi [prescribing information]. Warren, NJ. PTC Theraputics Inc.; November 2024.
2. A Study of SmartFlow Magnetic Resonance (MR) Compatible Ventricular Cannula for Administering Eladocagene Exuparvovec to Pediatric Participants. National Library of Medicine. 2025 Apr 10 [cited 2025 May 3]. Available from: https://clinicaltrials.gov/study/NCT04903288?intr=%20Eladocagene%20Exuparvovec&rank=1#study-overview
3. Aromatic L-Amino Acid Decarboxylase Deficiency. National Organization for Rare Disorders (NORD). 2024 November 14 [cited 2025 May 3]. Available from: https://rarediseases.org/rare-diseases/aromatic-l-amino-acid-decarboxylase-deficiency/#disease-overview-main

This policy will be revised as necessary and reviewed no less than annually.

Devised: 5/13/25

Revised:

Reviewed:

MA UM Committee approval: 6/9/25

DHS PARP approval: 6/24/25