Mass General Brigham
Health Plan

Medical Policy
Kebilidi (eladocagene exuparvovec-tneq)

Policy Number: 109

Contents

Overview

Kebilidi is an in vivo gene therapy treatment developed to target aromatic L-amino acid decarboxylase (AADC) deficiency's underlying cause using adeno-associated virus serotype 2 (AAV2)-based viral vectors to deliver functional copies of the DDC gene directly into a part of the brain that produces dopamine. This increases expression of AADC and, by extension, dopamine production, in patients whose AADC deficiency is genetic.

FDA-Approved Indication:

- Treatment of adult and pediatric patients with AADC deficiency.

Medicare Variation

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) for medical necessity determinations for its Medicare Advantage plan members. National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs), and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, Mass General Brigham Health Plan's medical policies are used for medical necessity determinations. **At the time of Mass General Brigham Health Plan's most recent policy review, Medicare had:**

- Medicare Benefit Policy Manual Chapter 15: Covered Medical and Other Health Services

When CMS documentation references FDA labeling, Mass General Brigham Health Plan develops coverage criteria to clarify medical necessity of the requested services. Mass General Brigham Health Plan coverage criteria align with FDA labeling without contradicting existing determinations and enhance the clarity of medical necessity requirements, documentation requirements, and clinical indications.

Criteria

The member meets all of the following criteria:

1. The member is at least 16 months of age; and
2. The member has a diagnosis of AADC deficiency confirmed by both of the following:
    a. Biallelic pathogenic variants in the DDC gene; and
    b. Decreased AADC enzyme activity in the member's plasma; or decreased levels of 5-HIAA, NV, and MHPG and increased levels of 3-OMD, L-Dopa, and 5-HTP in the member's cerebrospinal fluid; and
3. The member has achieved skull maturity sufficient for stereotaxis as documented in a radiology report; and
4. The member has not received any prior gene therapy for AADC deficiency; and
5. The medication is prescribed by or in consultation with a neurologist or neurosurgeon; and

Dosage and Administration

1. For single-dose intraputaminal infusion only.
2. Kebilidi is administered in a single stereotactic neurosurgical procedure as per the recommended dose:
    a. Total recommended dose is 1.8x1011 vg (0.32 mL)
    b. Total number of infusions is four:
        i. Two in anterior putamen; and
        ii. Two in posterior putamen.
    c. Volume (dose) per infusion is 0.08 mL (0.45x1011 vg)
    d. Infusion rate at each target point is 0.003 mL/min
    e. Dose duration for infusion at each target point is 27 minutes

Mass General Brigham ACO Variation

Prior Authorization Required	Yes ☒ No ☐

Prior authorization requests for Kebilidi for Mass General Brigham ACO members should be submitted to the MassHealth Drug Utilization Review Program. Criteria for Kebilidi are found in Table 65: Enzyme and Metabolic Disorder Therapies.

One Care and Senior Care Options Variation

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from CMS for medical necessity determinations for its One Care and SCO plan members. NCDs, LCDs, LCAs, and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, or the member does not meet the medical necessity criteria for the requested service, Mass General Brigham Health Plan uses medical necessity guidelines from MassHealth. **See Medicare Advantage criteria and exclusions, above. If Medicare Advantage criteria are not met, then MassHealth criteria are applied.**

Commercial and Qualified Health Plans Variation

Prior Authorization Required	Yes ☒ No ☐

Prior authorization for Kebilidi for Commercial and Qualified Health Plan members is managed by Prime Therapeutics. See the Prime Therapeutics policy for Kebilidi for more information.

Codes

The following codes are included below for informational purposes only; inclusion of a code does not constitute or imply coverage or reimbursement.

Authorized Code	Code Description
J3590	Unclassified biologics

Summary of Evidence

Eladocagene exuparvovec (Kebilidi) is the first disease-modifying treatment for AADC deficiency, a rare neurometabolic disorder characterized by profound motor delay, oculogyric crises, autonomic dysfunction, and developmental impairment. Regulatory assessments, including the NICE HST26 guidance (2023), conclude that the therapy offers clinically meaningful benefits despite high upfront costs, supported by evidence from open-label trials and long-term follow-up studies. Peer-reviewed clinical data demonstrate substantial and durable improvement in motor function.

In a multicenter analysis, Hwu et al. (2025) reported that children treated with eladocagene exuparvovec achieved meaningful gains on the Peabody Developmental Motor Scales-2 (PDMS-2), with improvements correlating strongly with Bayley-III motor composite scores, and the attainment of major motor milestones such as head control and independent sitting. Long-term follow-up (up to 5 years) described by Tai et al. (2022) similarly showed sustained motor improvement, reduced frequency of oculogyric crises, and gains in functional abilities, with an overall safety profile primarily characterized by transient postoperative dyskinesias and manageable adverse events. Expert guidance further emphasizes the critical role of structured, multidisciplinary rehabilitation after gene therapy. Position statements by Lee et al. (2024) and safety/implementation recommendations by Roubertie et al. (2024) highlight the need for standardized post-treatment physical therapy, careful perioperative management, and long-term neurodevelopmental monitoring to maximize benefit and support evidence generation.

Health technology assessments including Hayes (2024) and NICE (2023) conclude that despite the rarity of AADC deficiency and limitations of small trial populations, the observed magnitude of functional gain is larger than expected with supportive care alone. Overall, across clinical trials, regulatory reviews, and expert consensus statements, the evidence supports eladocagene exuparvovec as a therapy that enables meaningful motor development and improved quality of life in a population with otherwise minimal therapeutic options.

Effective Dates

April 2026: Effective date.

References

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