Kebilidi (eladocagene exuparvovec-tneq)

Humana.
Medicaid Medical Coverage Policy

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State: VA

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Description

A rare, autosomal recessive neurometabolic condition, aromatic l-amino acid decarboxylase (AADC) deficiency is characterized by decreased synthesis of serotonin and catecholamines (adrenaline, dopamine and norepinephrine).[5] This deficiency results from mutations in the dopa decarboxylase (*DDC*) gene, which codes for the AADC enzyme. This enzyme is responsible for the final step of the synthesis of dopamine and serotonin.[2] Developmental delay, hypotonia (low muscle tone) and oculogyric crises (involuntary upward deviation of the eyes) are common in infants with AADC deficiency. Other characteristics of the condition include autonomic dysfunction, disturbances in mood and sleep and intellectual impairment.[1,5] Parents of AADC deficiency individuals typically do not exhibit symptoms of the illness, although they each contain one copy of the harmful mutant gene. For individuals with this disease, symptoms usually appear within the first year of life and need lifetime care.[2]

In Taiwan, where the syndrome is more prevalent (due to a founder mutation in the population), individuals have an almost uniformly severe disease progression with significant motor impairment and a high risk of death in early childhood. Internationally, the majority of reported individuals have severe symptoms and motor disability, however individuals with mild disease and more favorable response to medical treatment have also been reported.[5] Based on genetic testing and cerebrospinal fluid (CSF) research, the estimated birth rate of people with AADC deficiency in the US is 1 to 3 per 100,000 live infants.[2]

AADC deficiency has no known cure, and the disease treatment methods currently used are supportive and symptomatic. Dopamine agonists, monoamine oxidase (MAO) inhibitors and vitamin B6 (a cofactor of AADC) are commonly used medications. Supportive interventions include occupational, physical and speech therapies, as well as feeding and nutritional treatments. Optimal regimens are difficult to achieve and response to therapy varies widely. AADC deficiency has limited treatment options, particularly those targeting its underlying cause. **Kebilidi (eladocagene exuparvovec-tneq)** is a gene therapy that has been developed as a potential approach.[2]

Kebilidi (eladocagene exuparvovec-tneq) is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of adult and pediatric individuals with AADC deficiency. This gene therapy is designed to deliver a copy of the *DDC* gene which encodes the AADC enzyme. Administration consists of four intraputaminal (into the brain region, called the putamen) infusions in a single stereotactic neurosurgical procedure, causing the putamen to express the AADC enzyme and subsequently produce dopamine.[7]

Coverage Determination

Refer all requests or questions regarding Kebilidi (eladocagene exuparvovec-tneq) to the Corporate Transplant Department.

Phone	***Fax***	***Email***
1-866-421-5663	*502-508-9300*	*transplant@humana.com*

Humana members may be eligible under the Plan for a *single, one-time lifetime dose* of **Kebilidi (eladocagene exuparvovec-tneq)** when the following requirements are met:

- Absence of limitations; **AND**
- Individual is 16 months through 10 years of age[7]; **AND**
- Confirmation of AADC deficiency due to biallelic mutations in the *DDC* gene[1,3,7]

Coverage Limitations

Humana members may **NOT** be eligible under the Plan for **Kebilidi (eladocagene exuparvovec-tneq)** for any indications other than those listed above including, but may not be limited to[1,7]:

- Individual has not achieved skull maturity assessed by neuroimaging; **OR**
- Individual has desire to become pregnant/reproduce **OR** unwilling to use effective contraception; **OR**
- Individual is pregnant or breastfeeding

A review of the current medical literature shows that there is **no evidence** to determine that this service is standard medical treatment. There is an absence of current, widely-used treatment guidelines or acceptable clinical literature examining benefit and long-term clinical outcomes establishing the value of this service in clinical management for these indications.

Coding Information

Any codes listed on this policy are for informational purposes only. Do not rely on the accuracy and inclusion of specific codes. Inclusion of a code does not guarantee coverage and/or reimbursement for a service or procedure.

CPT® Code(s)	Description	Comments
64999	Unlisted procedure; nervous system when specified as administration of gene therapy	
CPT® Category III Code(s)	**Description**	**Comments**
No code(s) identified		
HCPCS Code(s)	**Description**	**Comments**
C9399	Unclassified drugs or biologicals	
J3490	Unclassified drugs	
J3590	Unclassified biologics	
ICD-10 Procedure	**Description**	**Comments**
XW0Q316	Introduction of eladocagene exuparvovec into cranial cavity and brain, percutaneous approach, new technology group 6	

References

1. ClinicalKey. Drug Monograph. Eladocagene exuparvovec. https://clinicalkey.com. Published January 13, 2025.

2. Hayes, Inc. Emerging Technology Report. Eladocagene Exuparvovec-tneq (Kebilidi; PTC Therapeutics) for aromatic l-amino acid decarboxylase deficiency. https://evidence.hayesinc.com. Published November 18, 2024.

3. IBM Micromedex. DrugPoint Summary. Eladocagene Exuparvovec-tneq https://micromedexsolutions.com. Updated December 11, 2024.

4. Lee HM, Mercimek-Andrews S, Horvath G, et al. A position statement on the post gene-therapy rehabilitation of aromatic l-amino acid decarboxylase deficiency patients. *Orphanet J Rare Dis*. 2024;19(1):17.

5. Pearson TS, Gilbert L, Opladen T, et al. AADC deficiency from infancy to adulthood: symptoms and developmental outcome in an international cohort of 63 patients. *J Inherit Metab Dis*. 2020;43(5):1121-1130.

6. 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.

7. US Food & Drug Administration (FDA). Kebilidi (eladocagene exuparvovec-tneq) prescribing information. https://fda.gov. Published November 2024.

Change Summary

03/04/2025 New Policy.