GOLD COAST HEALTH PLAN TOTAL Care ADVANTAGE℠

TOTAL CARE ADVANTAGE PART B CLINICAL GUIDELINES

KEBILIDI™ *(eladocagene exuparvovec-tneq)* susp, for intraputaminal infusion

PA Criteria	Criteria Details
Description	KEBILIDI™ is an adeno-associated virus (AAV) vector-based gene therapy that expresses the human aromatic L-amino acid decarboxylase enzyme (AADC).
Covered Uses (FDA approved indication)	KEBILIDI is indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency*. **This indication is approved under accelerated approval based on change from baseline in gross motor milestone achievement at 48 weeks post-treatment. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.*
Dosing and Administration	See table below

Indication	Dosing Regimen	Maximum Dose
AADC deficiency	Recommended dose: 1.8 x 10^{11} vector genomes (0.32 mL) Medication is administered as a single-dose **intraputaminal** infusion. • 1 dose = Four 0.08 mL infusions (0.45 x 10^{11} vg each) » 2 infusions per putamen (anterior and posterior) » Administer at rate of **0.003 mL/min** each » Total 27 minutes per site • Administered in a single stereotactic surgery using FDA approved cannula for intraparenchymal infusion KEBILIDI is administered at designated treatment centers that specialize in pediatric neurosurgery. • Texas Children's Hospital (Houston, TX) • Boston Children's Hospital (Boston, MA)	1.8 x 10^{11} vector genomes

Billing and Coding Information

10-digit NDC	11-digit NDC
Package (carton): 52856-601-01 Container (vial): 52855-601-11	Package (carton): 52856-0601-01 Container (vial): 52855-0601-11

HCPCS Code	Description
C9399	Unclassified drugs or biologicals
J3590	Unclassified biologics

CPT Procedural Codes	Description
64999	Unlisted procedure, nervous system

Product Availability	*Single-dose vial:* 2.8 x 10^{11} vg/0.5 mL vial - intraputaminal administration ONLY.
Contraindications	Patients who have NOT achieved skull maturity assessed by neuroimaging.
Recommended Medical Monitoring	KEBILIDI has been associated with: • Dyskinesia • Procedural complications » Respiratory arrest » Cardiac arrest » CSF leak » Intracranial bleeding » Neuroinflammation » Acute infarction » Infection There is currently NO clinical data from the use of KEBILIDI in pregnant women or during lactation. However, it is recommended to verify negative pregnancy status in females with reproductive potential prior to administering KEBILIDI.
Approval Criteria	a. Physician administered intraputaminal infusion via stereotactic surgery b. AADC Deficiency **(must meet all)**: i. Diagnosis of AADC deficiency by documentation of positive testing from **two of the following** core diagnostic tests: 1. CSF neurotransmitter metabolite panel 2. Single gene or genetic panel testing 3. Plasma enzyme assay ii. Patient is experiencing persistent neurological defects secondary to AADC deficiency despite standard medical therapy (see Appendix) iii. Prescribed by or in consultation with a neurologist and/or geneticist iv. Age ≥ 16 months old v. Documentation that patient has achieved skull maturity via neuroimaging vi. Documentation of baseline laboratory tests demonstrating anti-AAV2 neutralizing antibody titer < 1200 fold or ELISA optical density (OD) > 1 vii. Dose does not exceed 1.8 x 1011 vector genomes (0.32 mL total volume)
Age Restriction	Age ≥ 16 months old – must confirm skull maturity (assessed via neuroimaging). KEBILIDI has NOT been studied in patients < 16 months old or ≥ 65 years old.
Coverage Duration	Total approval duration: one-time approval ONLY **(maximum one dose per lifetime)** Dose will be approved according to the FDA approved labeling or within accepted standards of medical practice.

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Appendix

Classic Clinical Symptoms of AADC Deficiency (per 2017 iNTD Guidelines)	Core Diagnostic Tools for Identifying AADC Deficiency (per 2017 iNTD Guidelines)
a. Movement disorders • Hypotonia • Dystonia • Dyskinesia • Tremor • Myoclonus • Oculogyric crisis • Hypokinesia b. Developmental Delay • Delayed motor development • Delayed speech development • Delayed cognitive development c. Tone Regulation • Floppy infant syndrome • Hypotonia • Hypertonia • Poor head control	There are three core diagnostic tools for identifying AADCD: 1. Low CSF level of 5-HIAA, HVA and MHPG with normal CSF pterins and increased levels of LDopa, 3-OMD and 5-HTP 2. Genetic diagnosis showing compound heterozygous or homozygous disease-causing variants in the DDC gene 3. Decreased AADC enzyme activity in plasma To confirm diagnosis of AADC deficiency, genetic testing should be completed and two of the three core diagnostic tests should be POSITIVE. If local resources allow, it is recommended to perform all three key diagnostic tests.

Core Recommendations for Treatment of AADC Deficiency (per 2017 iNTD Guidelines)

The core recommendations for treatment of AADCD are below - In general, multiple drug classes will be needed:

- First line treatment with selective dopamine agonists, MAO-inhibitors, and pyridoxine.
- Additional symptomatic treatment agents with anticholinergic agents, melatonin, benzodiazepines, and alpha-adrenoreceptor blockers.

STATUS	DATE REVISED	REVIEW DATE	APPROVED/REVIEWED BY	EFFECTIVE DATE
Created	9/19/2025	9/19/2025	Tamara Chinarian, PharmD, Clinical Pharmacist	N/A
Approved	N/A	11/13/2025	Pharmacy & Therapeutics (P&T) Committee	11/13/2025