

Prior Authorization DRUG Guidelines

KEBILIDI® (Eladocagene exuparvovec-tneq)

Effective Date:

Date Developed: 12/5/24 by Howard Taekman MD

Date Approved by P&T Committee:

KEBILIDI is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

Authorization Criteria:

1. For single dose intraputaminial infusion only.
2. Recommended dose: 1.8×10^{11} vector genomes (vg).
3. Administer a total dose of 1.8×10^{11} vg (0.32 mL total volume) delivered as four 0.08 mL (0.45×10^{11} vg) infusions (two sites per putamen-anterior and posterior) at a rate of 0.003 mL/minute (0.18 mL/hour) for a total of 30-27 minutes per site, administered in a single stereotactic surgery using a 31 cannula that is FDA-authorized for intraparenchymal infusion (i.e., ClearPoint SmartFlow Neuro Cannula Part Number NGS-NC-01-EE or NGS-NC-02-EE).
4. Administered in a medical center that specializes in stereotactic neurosurgery

CONTRAINDICATIONS:

1. The safety and efficacy of KEBILIDI have not been studied in pediatric patients younger than 16 months or adults 65 years of age and older.
2. Pregnant women.

REFERENCES

FDA Approves First Gene Therapy for Treatment of Aromatic L-amino Acid Decarboxylase Deficiency. U.S. Food & Drug Administration (FDA), (2024, November 14).
<https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-therapy-treatment-aromatic-l-amino-acid-decarboxylase-deficiency>

Kebilidi [prescribing information]. Warren, NJ: PTC Therapeutics, Inc.; (2024, November).
<https://www.fda.gov/media/183530/download?attachment>

KEBILIDI. U.S. Food & Drug Administration (FDA), (2024, November 14).
<https://www.fda.gov/vaccines-blood-biologics/kebilidi>

Anonymous, 2024. PTC Therapeutics Announces FDA Approval of AADC Deficiency Gene Therapy. *PTC Therapeutics*. Retrieved December 9, 2024, from <https://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-announces-fda-approval-aadc-deficiency-gene>.

A. Attachments: None

B. History:

Policy created by Howard Taekman, MD; Robert Sterling MD

Date Approved by P&T Committee: 2/18/25

Date Reviewed/No Updates:

Revision Date	Content Revised (Yes/No)	Contributors	Review/Revision Notes