

An innovative treatment for people with AADC deficiency

Kebilidi™
(eladocagene exuparvovec-tneq)

FIRST AND ONLY gene therapy
approved for children and adults
with aromatic L-amino acid
decarboxylase (AADC) deficiency¹

Find more support for your
AADC deficiency journey at
[KEBILIDI.com](https://www.kebilidi.com)



IMPORTANT SAFETY INFORMATION

What is KEBILIDI™ used for?

KEBILIDI is a gene therapy indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).

About KEBILIDI™ (eladocagene exuparvovec-tneq) and AADC deficiency

KEBILIDI is a gene therapy designed to help restore AADC enzyme activity.¹

AADC deficiency is a rare genetic disorder that affects the brain, causes weak muscle tone, and impacts how a child develops.² It is caused by a change in the gene that controls the production of AADC (also called dopa decarboxylase or DDC). The condition prevents development of a child's nervous system, which means that many of the body's functions do not develop completely during childhood³:

Body functions that may not fully develop due to AADC deficiency³:



Movement and coordination

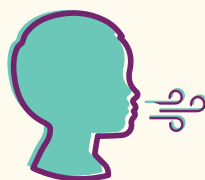
- Head control
- Sitting
- Standing
- Walking



Mental ability



Eating



Breathing



Speech

IMPORTANT SAFETY INFORMATION (cont'd)

What Warnings should I know about KEBILIDI?

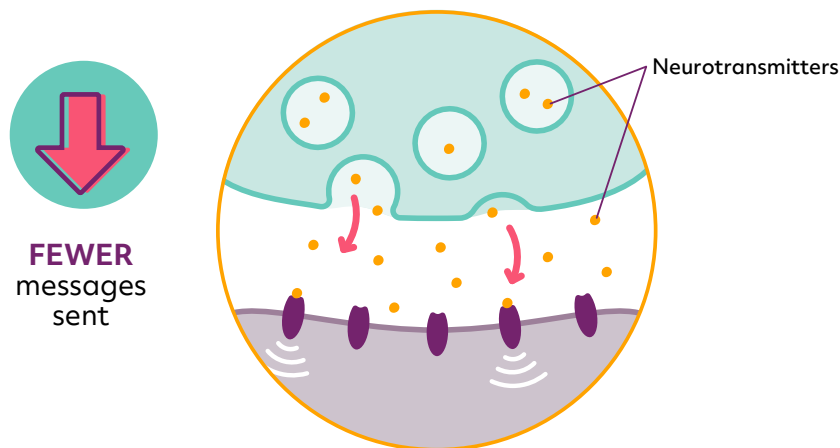
Procedural complications can occur with administration of KEBILIDI, which can include stopping of breathing or stopping of the heart within 24 hours of the procedure and during post-surgical care. Potential additional procedure related adverse events include cerebrospinal fluid leak, bleeding within the brain, inflammation of brain tissue, damage of brain tissue, and infection.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).

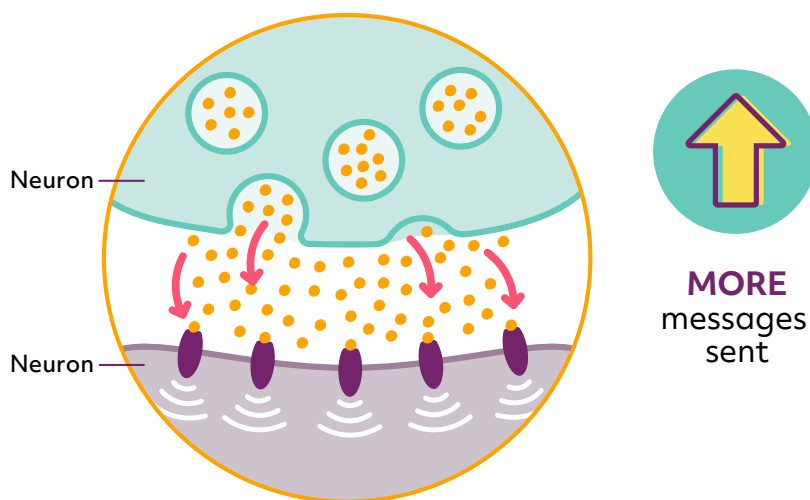


KEBILIDI helps restore dopamine production by editing the missing gene¹

People with AADC deficiency have a change in their genes that leads to lower production of the AADC enzyme.¹



KEBILIDI provides a working copy of the *DDC* gene to help cells produce the AADC enzyme. This enzyme allows the body to produce important brain chemicals, like dopamine, that are needed for typical functioning.¹



IMPORTANT SAFETY INFORMATION (cont'd)

What Warnings should I know about KEBILIDI? (cont'd)

Uncontrolled movement was reported after administration of KEBILIDI. All events were reported within 3 months of administration and 2 events required hospitalization. Uncontrolled movements in the face, arm, leg, or entire body should be reported right away to a healthcare provider.

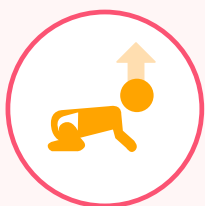
Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).

Kebilidi[™]
(eladocagene exuparvec-tneq)

KEBILIDI™ (eladocagene exuparvec-tneq) helped children achieve major motor milestones¹

KEBILIDI was studied in a clinical trial of 13 individuals aged 16 months to 10 years with severe AADC deficiency who had achieved skull development. The study measured the motor milestones these individuals were able to achieve 48 weeks (about 11 months) after treatment with KEBILIDI. One individual did not complete the trial.

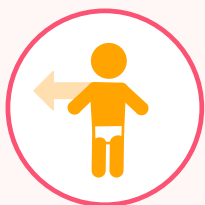
**For individuals without physical milestones when the clinical trial began
In less than 1 year after treatment, some individuals achieved:**



Full head control
3 patients



Sitting with or without assistance
2 patients



Walking backward
2 patients

Milestones
were measured
using the Peabody
Developmental
Motor Scales
second edition
(PDMS-2)

IMPORTANT SAFETY INFORMATION (cont'd)

You should not use KEBILIDI if:

skull maturity has not been achieved when assessed by neuroimaging, since skull maturity is needed for administration of KEBILIDI.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).


Kebilidi™
(eladocagene exuparvec-tneq)

67% of individuals with severe AADC deficiency achieved a new motor milestone after KEBILIDI treatment¹



For those **NOT TREATED**
with KEBILIDI, **zero major motor milestones**
were achieved when they were evaluated by doctors.

Peabody Developmental Motor Scales second edition (PDMS-2)
(a tool used by healthcare providers to assess motor skills)⁴

Full head control	Holds head for 8 seconds while rotating to follow toy
Sitting without assistance	Sits unsupported for 60 seconds
Sitting assisted	Maintains balance for 8 seconds while extending arms and hands
Walking backward	Walks backward 5 steps

IMPORTANT SAFETY INFORMATION (cont'd)

What are the side effects of KEBILIDI?

The most common side effects of KEBILIDI are uncontrolled movement, fever, low blood pressure, low red blood cell count, excess saliva production, low blood potassium,

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).

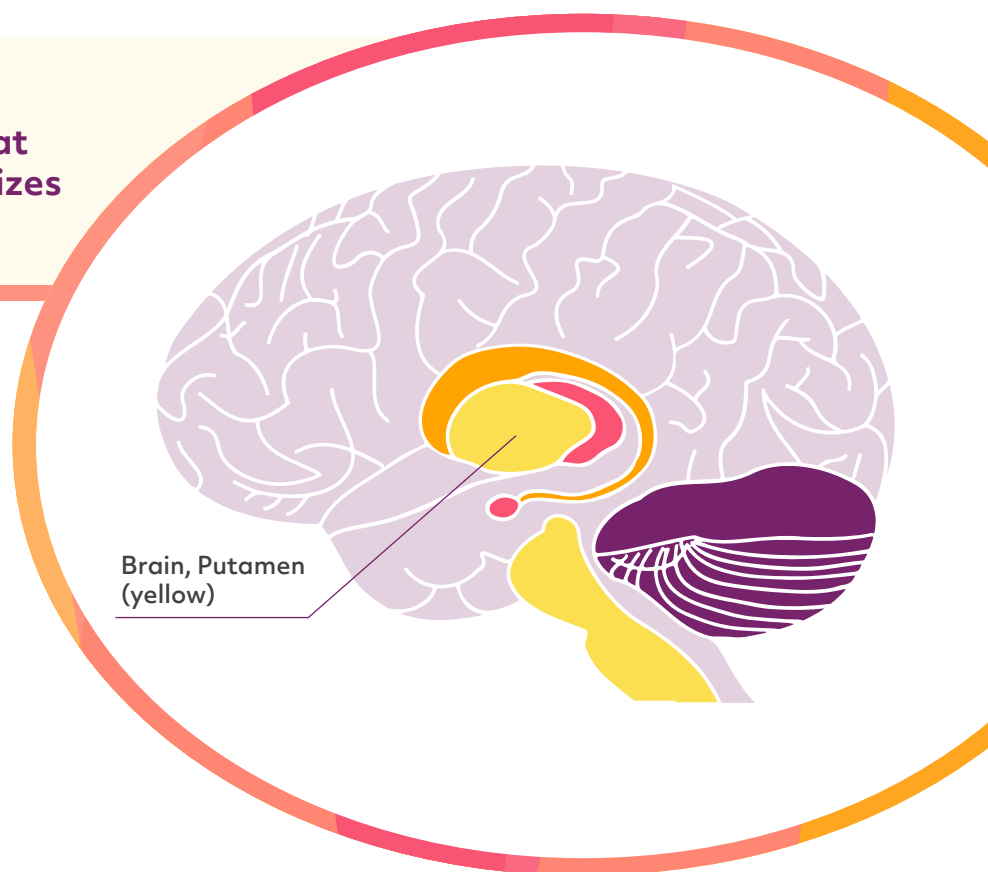

Kebilidi[™]
(eladocagene exuparvovec-tneq)

How is KEBILIDI™ (eladocagene exuparvovec-tneq) delivered?

KEBILIDI is a one-time gene therapy that is delivered directly into the area of the brain (called the putamen) where the AADC enzyme is made.¹

KEBILIDI is delivered using a one-time surgical procedure at a medical center that specializes in stereotactic neurosurgery.

KEBILIDI uses a modified virus, called an adeno-associated virus, to deliver a healthy copy of the *DDC* gene into brain cells. The therapy is delivered through two small holes in the skull during minimally invasive neurosurgery.¹



Why is it delivered to that part of the brain?

The putamen is targeted because it is the area **responsible for motor control, learning, behaviors, and emotions.**⁵

IMPORTANT SAFETY INFORMATION (cont'd)

What are the side effects of KEBILIDI? (cont'd)

low blood phosphate, sleeplessness, low blood magnesium, and procedural complications, including stopping of breathing and stopping of the heart.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).


Kebilidi[™]
(eladocagene exuparvovec-tneq)

What can we expect before, during, and after treatment with KEBILIDI?^{1,3}



Brain scans

Before treatment begins, **detailed brain images** are taken to help the medical team properly plan.



Hospital admission

Your team will **provide information** on length of stay.



Minimally invasive neurosurgery

On the day of surgery, the neurosurgeon will make two small holes in the skull. KEBILIDI will be infused through special tubes into the brain, in an area called the putamen. **The tubes are used to deliver KEBILIDI by four infusions into the putamen**—two on the left side and two on the right side of the brain.



Post surgery

After the surgery, the team will conduct brain imaging and monitor for any potential complications. Recovery may include overnight stays in the hospital. Follow-up with the care team may include **lab tests, cognitive tests, and physical and occupational therapy** to help develop motor skills.



After surgery care

The adenovirus used to deliver KEBILIDI will be present in bodily secretions (nasal mucous, urine, stools) for 3 weeks. Proper handling using gloves and storage/disposal of these materials will be discussed by your care team.

IMPORTANT SAFETY INFORMATION

What is KEBILIDI™ used for?

KEBILIDI is a gene therapy indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).


Kebilidi[™]
(eladocagene exuparvovec-tneq)

Understanding side effects and complications

In clinical studies of KEBILIDI™ (eladocagene exuparvovec-tneq), the most common adverse reactions, reported in 15 percent or more of patients, are listed in the table below.¹

Adverse reaction or side effect	What it means	Patients treated with KEBILIDI, N=13 (%)
Dyskinesia*	Involuntary movements of face, arm, leg, or entire body	10 (77%)
Pyrexia	Fever	5 (38%)
Hypotension	Low blood pressure	4 (31%)
Anemia	Fewer red blood cells than typical	4 (31%)
Salivary hypersecretion	Increased saliva	3 (23%)
Hypokalemia	Lower levels of potassium in the blood	3 (23%)
Hypophosphatemia	Lower levels of phosphate in the blood	3 (23%)
Insomnia	Difficulty sleeping	3 (23%)
Hypomagnesemia	Low levels of magnesium	2 (15%)
Procedural complications	Effects from the surgery, including respiratory and cardiac arrest	2 (15%)

*All dyskinesia events were reported within 3 months of administration, and two events required hospitalization. Your doctor may provide medication (called dopamine antagonists) to help control these involuntary movements.

NOTE: Patients should be closely monitored for procedure-related complications and risks associated with general anesthesia before, during, and after surgery.

IMPORTANT SAFETY INFORMATION (cont'd)

What Warnings should I know about KEBILIDI?

Procedural complications can occur with administration of KEBILIDI, which can include stopping of breathing or stopping of the heart within 24 hours of the procedure and during post-surgical care.

Please see additional Important Safety Information on [page 9](#) and the [Full Prescribing Information](#).



IMPORTANT SAFETY INFORMATION

What is KEBILIDI™ used for?

KEBILIDI is a gene therapy indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

What Warnings should I know about KEBILIDI?

Procedural complications can occur with administration of KEBILIDI, which can include stopping of breathing or stopping of the heart within 24 hours of the procedure and during post-surgical care. Potential additional procedure related adverse events include cerebrospinal fluid leak, bleeding within the brain, inflammation of brain tissue, damage of brain tissue, and infection.

Uncontrolled movement was reported after administration of KEBILIDI. All events were reported within 3 months of administration and 2 events required hospitalization. Uncontrolled movements in the face, arm, leg, or entire body should be reported right away to a healthcare provider.

You should not use KEBILIDI if:

skull maturity has not been achieved when assessed by neuroimaging, since skull maturity is needed for administration of KEBILIDI.

What are the side effects of KEBILIDI?

The most common side effects of KEBILIDI are uncontrolled movement, fever, low blood pressure, low red blood cell count, excess saliva production, low blood potassium, low blood phosphate, sleeplessness, low blood magnesium, and procedural complications, including stopping of breathing and stopping of the heart.

To report an adverse event, please call 1-866-562-4620 or email at usmedinfo@ptcbio.com. You may also report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.

Please see www.KEBILIDI.com for the full Prescribing Information for additional information.

References: 1. KEBILIDI™ Prescribing Information. PTC Therapeutics. 2024. 2. Wassenberg T, Molero-Luis M, Jeltsch K, et al. Consensus guideline for the diagnosis and treatment of aromatic L-amino acid decarboxylase (AADC) deficiency. *Orphanet J Rare Dis*. 2017;12(1):12. Published 2017 Jan 18. doi:10.1186/s13023-016-0522-z. 3. Chien YH, Lee NC, Tseng SH, et al. Efficacy and safety of AAV2 gene therapy in children with aromatic L-amino acid decarboxylase deficiency: an open-label, phase 1/2 trial. *Lancet Child Adolesc Health*. 2017;1(4):265-273. doi:10.1016/S2352-4642(17)30125-6. 4. Folio MR, Fewell RR. *Peabody Developmental Motor Scales: Examiner's Manual*. 2nd ed. Austin, TX: Pro-Ed; 2000. 5. Hwu PW, Kiening K, Anselm I, et al. Gene therapy in the putamen for curing AADC deficiency and Parkinson's disease. *EMBO Mol Med*. 2021;13(9):e14712.



ENROLL IN PTC CARES™ TO
GET CONNECTED WITH YOUR
DEDICATED CASE MANAGER

SUPPORT FOR THE TREATMENT JOURNEY

PTC Cares is a support program staffed by Case Managers who help individuals living with rare disorders and their caregivers by coordinating treatment and guiding them through it. Our goal is to make the process of getting treatment easier for you and your family. After you enroll in PTC Cares, you'll be provided with a dedicated Case Manager who will coordinate treatment and be there to support your family through your journey.

Eligible individuals may have access to:



Connection to patient
advocacy organizations



Travel
assistance



Support from a dedicated
Case Manager

Visit PTCCares.com/aadc to learn more
about AADC deficiency resources

Have questions?



We're always ready to help!

1-844-4PTC-CARES (1-844-478-2227)

M – F, 8 AM – 6 PM ET



© 2026 PTC Therapeutics, Inc. All rights reserved.
PTC Cares, PTC Cares Logo, and KEBILIDI are trademarks of
PTC Therapeutics, Inc. or affiliates.
US-KEB-0254 | 08/26