



CAREGIVER
SUPPORT

POST-TREATMENT CARE TEAM

Post-treatment care of your child following Upstaza™▼ (eladocagene exuparvovec) gene therapy for AADC deficiency is essential for monitoring recovery and promoting developmental improvements.^{1,2}

It requires a coordinated approach to implement a post-treatment care plan that will help your child maximise their outcomes with Upstaza gene therapy.^{1,2}



Local healthcare professionals
(referring neurologist and local physician)¹⁻³

Local team:

After gene therapy and post-procedure approval, your child will return home to their referring neurologist and local care team for post-treatment care.^{1,2}

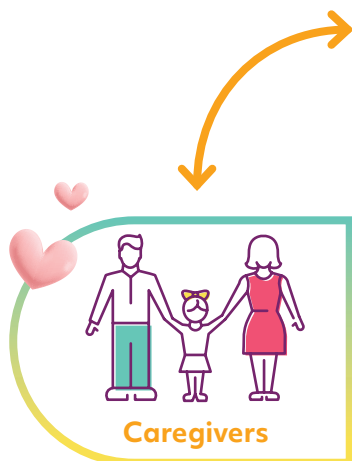
Treatment centre:

Specialised in stereotactic neurosurgery, this is the centre where your child's gene therapy surgery took place.^{1,3}



Neurosurgeon
(manages the neurosurgical team)^{1,3}

Post-treatment care
multidisciplinary
team²



Caregivers



The Upstaza Patient Information Leaflet can be accessed via the QR code (page 24 of the document); a printed version is available from your local healthcare professional. If your child gets any side effects, talk to your doctor, pharmacist or nurse. This includes any side effects not listed in the Patient Information Leaflet. By reporting side effects, you help provide more information on the safety of this medicine.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you or your child may get via the national reporting system. Side effects should also be reported to PTC Therapeutics at pharmacovigilance@ptcbio.com.

This material was funded by PTC Therapeutics International Ltd and is only intended for caregivers of patients with aromatic L-amino acid decarboxylase (AADC) deficiency who have been prescribed Upstaza gene therapy.

GL-UPZ-0510 | August 2024

PTC
THERAPEUTICS

Your post-treatment care multidisciplinary team

The team caring for your child may change over time as their needs and abilities evolve;^{2,3} however, you will always remain at the centre of your child's post-treatment care.



Local healthcare professionals (referring neurologist and local physician)

Your main points of contact for post-treatment care after leaving the treatment centre.¹⁻³



Name: _____

Name: _____

Phone: _____

Phone: _____

Email: _____

Email: _____

Treatment centre neurosurgeon



Responsible for monitoring post-surgery recovery, they will work with the referring neurologist to lead the post-treatment care multidisciplinary team^{1,3}

Caregiver(s)



Cares for the patient recovering from treatment and ensures the post-treatment care plan is implemented²

Physical therapist or occupational therapist



Gives physical therapy guidance and performs functional tests¹⁻⁴

Psychologist or social worker



Provides psychological care addressing the challenges of caregivers and children with rare movement disorders¹⁻⁴

Speech therapist



Facilitates and monitors language development^{1,2,4}

Dietitian or gastroenterologist



Provides support with feeding difficulties^{1,2,4}

Caregivers play a crucial role in the post-treatment care of patients with AADC deficiency after Upstaza gene therapy.² You can support your child by providing opportunities for them to develop new skills, giving them the freedom to try functional activities, such as self-feeding and incorporating play and in-home therapy into daily routines.² You can also explore other therapy strategies that will suit their post-treatment goals and impairments, such as music therapy, equine-assisted therapy, aquatic therapy, cognitive and communication training, or sensory-based therapy.² Speak with your local healthcare professional to find out more about therapy options in your area.

Caring for someone with AADC deficiency can be challenging, but it's important to remember that the post-treatment care multidisciplinary team is intended to support both you and your child.

There are also various support groups available online, including [teachrare.org](https://www.teachrare.org) and www.aadcresearch.org.

This material is not intended to be used to change an existing post-treatment care plan developed by healthcare professionals. All post-treatment care performed at home should align with advice from your child's healthcare professionals and you should consult them if you have questions about your child's unique post-treatment care plan.

AADC, aromatic L-amino acid decarboxylase.

References

1. Upstaza (eladocagene exuparvovec) Summary of Product Characteristics. PTC Therapeutics. 2024.
2. Lee H-M, et al. *Orphanet J Rare Dis*. 2024;19(1):17.
3. Roubertie A, et al. *J Inher Metab Dis*. 2024;47(3):463-475.
4. Wassenberg T, et al. *Orphanet J Rare Dis*. 2017;12(1):12.

