

## Eladocogene Exuparvovec: Adis Evaluation

### Key Points

- A gene therapy developed by PTC Therapeutics for the treatment of AADC deficiency
- Received its first approval on 20 July 2022 in the EU
- Approved for use in patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of AADC deficiency with a severe phenotype (i.e. who cannot sit, stand or walk)

### Summary

Eladocogene exuparvovec (Upstaza™) is a gene therapy developed by PTC Therapeutics for the treatment of human aromatic L-amino acid decarboxylase (AADC) deficiency.

Eladocogene exuparvovec comprises an adeno-associated virus vector that delivers the dopa decarboxylase (DDC) gene, the gene for human AADC.

Eladocogene exuparvovec was approved in July 2022 in the EU for the treatment of patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of AADC deficiency with a severe phenotype (i.e. patients who cannot sit, stand or walk).

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