EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 May 2022
EMA/CHMP/274542/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Upstaza

eladocagene exuparvovec

On 19 May 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Upstaza[3], intended for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency. As Upstaza is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies. The applicant for this medicinal product is PTC Therapeutics International Limited.

Upstaza will be available as a 2.8 x 10^{11} vector genomes/0.5 mL solution for infusion. The active substance of Upstaza is eladocagene exuparvovec, a gene therapy product which infuses the gene encoding for the human AADC enzyme into the putamen region of the brain. The subsequent expression of AADC results in dopamine production and, as a result, development of motor function in patients with AADC deficiency.

The benefit of Upstaza is the improvement in motor function. The most common side effects are initial insomnia, irritability and dyskinesia.

The full indication is:

> Upstaza is indicated for the treatment of patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of aromatic L-amino acid decarboxylase (AADC) deficiency with a severe phenotype (see section 5.1).

Upstaza should be administered by a qualified neurosurgeon in a centre specialised in stereotactic neurosurgery.

Detailed recommendations for the use of this product will be described in the summary of product

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.