17 October 2024
EMA/CHMP/474509/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Wainzua

eplontersen

On 17 October 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Wainzua[2], intended for the treatment of adults with hereditary transthyretin-mediated amyloidosis (ATTRv) and stage 1 or 2 polyneuropathy.

The applicant for this medicinal product is AstraZeneca AB.

Wainzua will be available as a 45 mg solution for injection in pre-filled pens. The active substance of Wainzua is eplontersen, an antisense oligonucleotide inhibitor (ATC code: N07XX21) that specifically binds to the human transthyretin (TTR) mRNA, leading to its degradation. As a result, both mutant and wild-type TTR proteins are no longer produced, which significantly reduces amyloid fibril deposits and greatly slows down the progression of ATTRv.

Wainzua has shown clinically relevant effects on both the neurological components of the disease and on quality of life. The most common side effects are vitamin A decreased and vomiting.

The full indication is:

> Wainzua is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (ATTRv) in adult patients with stage 1 or stage 2 polyneuropathy.

Treatment with Wainzua should be prescribed and supervised by a physician experienced in the treatment of patients with hereditary transthyretin-mediated amyloidosis (ATTRv).

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

[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] 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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