Eplontersen (hereditary transthyretin-mediated amyloidosis with stage 1 or 2 polyneuropathy)

Resolution of: 16 October 2025/13. January 2026 valid until: unlimited
Entry into force on: 16 October 2025/15. January 2026
Federal Gazette, BAnz AT 11.11.2025 B5/10.02.2026 B3

Therapeutic indication (according to the marketing authorisation of 06 March 2025):

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

Therapeutic indication of the resolution (resolution of 16 October 2025):

See therapeutic indication according to marketing authorisation.

1. Additional benefit of the medicinal product in relation to the appropriate comparator therapy

Adults with hereditary transthyretin-mediated amyloidosis with stage 1 or stage 2 polyneuropathy

Appropriate comparator therapy:

Vutrisiran

Extent and probability of the additional benefit of eplontersen compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:[1]

Adults with hereditary transthyretin-mediated amyloidosis with stage 1 or stage 2 polyneuropathy

There are no assessable data.

[1] Data from the dossier assessment of the Institute for Quality and Efficiency in Health Care (IQWiG) (A25-52) unless otherwise indicated.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ risk of bias	Summary
Mortality	n.a.	There are no assessable data.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	n.a.	There are no assessable data.
Side effects	n.a.	There are no assessable data.
Explanations: ↑: statistically significant and relevant positive effect with low/unclear reliability of data ↓: statistically significant and relevant negative effect with low/unclear reliability of data ↑↑: statistically significant and relevant positive effect with high reliability of data ↓↓: statistically significant and relevant negative effect with high reliability of data ↔: no statistically significant or relevant difference ∅: No data available. n.a.: not assessable		

2. Number of patients or demarcation of patient groups eligible for treatment

Adults with hereditary transthyretin-mediated amyloidosis with stage 1 or stage 2 polyneuropathy

Approx. 360 patients

3. Requirements for a quality-assured application

The requirements in the product information are to be taken into account. The European Medicines Agency (EMA) provides the contents of the product information (summary of product characteristics, SmPC) for Wainzua (active ingredient: eplontersen) at the following publicly accessible link (last access: 4 July 2025):

https://www.ema.europa.eu/en/documents/product-information/wainzua-epar-product-information_en.pdf

Treatment with eplontersen should only be initiated and monitored by specialists and general practitioners experienced in the treatment of patients with hereditary transthyretin-mediated amyloidosis.

4. Treatment costs

Annual treatment costs:

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Eplontersen	€ 391,590.42
Appropriate comparator therapy:	
Vutrisiran	€ 300,962.00

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 August 2025)

Costs for additionally required SHI services: not applicable

5. Designation of medicinal products with new active ingredients according to Section 35a, paragraph 3, sentence 4 SGB V that can be used in a combination therapy with the assessed medicinal product

In the context of the designation of medicinal products with new active ingredients pursuant to Section 35a, paragraph 3, sentence 4 SGB V, the following findings are made:

Adults with hereditary transthyretin-mediated amyloidosis with stage 1 or stage 2 polyneuropathy

- No medicinal product with new active ingredients that can be used in a combination therapy that fulfils the requirements of Section 35a, paragraph 3, sentence 4 SGB V.

The designation of combinations exclusively serves the implementation of the combination discount according to Section 130e SGB V between health insurance funds and pharmaceutical companies. The findings made neither restrict the scope of treatment required to fulfil the medical treatment mandate, nor do they make statements about expediency or economic feasibility.

6. Percentage of study participants at study sites within the scope of SGB V in accordance with Section 35a, paragraph 3, sentence 5 SGB V

The medicinal product eplontersen (Wainzua) is a medicinal product placed on the market from 1 January 2025.

The percentage of study participants in the clinical studies of the medicinal product conducted or commissioned by the pharmaceutical company in the therapeutic indication to be assessed who participated at study sites within the scope of SGB V (German Social Security Code) is < 5 per cent of the total number of study participants.

The clinical studies of the medicinal product in the therapeutic indication to be assessed were therefore not conducted to a relevant extent within the scope of SGB V.