Lanadelumab (reassessment of an orphan drug after exceeding the EUR 50 million turnover limit (hereditary angioedema, prevention, ≥ 12 years))

Resolution of: 4 November 2021 Valid until: unlimited
Entry into force on: 4 November 2021
BAnz AT 26 11 2021 B6

Therapeutic indication (according to the marketing authorisation of 22 November 2018):

Takhzyro is indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged 12 years and older.

Therapeutic indication of the resolution (resolution of 4 November 2021):

see therapeutic indication according to marketing authorisation

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

Adolescents and adults 12 years of age and older with recurrent attacks of hereditary angioedema

Appropriate comparator therapy of lanadelumab for routine prevention:

A routine prevention with C1 esterase inhibitor

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

An additional benefit is not proven.

Study results according to endpoints: [1]

No adequate data are available to allow an assessment of the additional benefit.

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.

[1] Data from the dossier assessment of the Institute for Quality and Efficiency in Health Care (IQWiG) (A21-63) unless otherwise indicated.

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
∅: There are no usable data for the benefit assessment.
n.a.: not assessable

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

Adolescents and adults 12 years of age and older with recurrent attacks of hereditary angioedema

approx. 140 – 430 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 Takhzyro (active ingredient: lanadelumab) at the following publicly accessible link (last access: 13 October 2021):
https://www.ema.europa.eu/en/documents/product-information/takhzyro-epar-product-information_en.pdf

Treatment with lanadelumab should only be initiated and monitored by doctors experienced in treating adolescent and adult patients with hereditary angioedema (HAE).

4. Treatment costs

Annual treatment costs:

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Lanadelumab	€ 191,757.54 - € 384,990.14
Appropriate comparator therapy:	
C1 esterase inhibitor[2]	€ 160,380.32 - € 213,781.87

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 October 2021)

Costs for additionally required SHI services: not applicable

[2] Different dosage information is shown in the product information of the C1-esterase inhibitors. The range shown is based on an administration every 3 - 4 days.