Resolution

Gemeinsamer Bundesausschuss

of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive (AM-RL): Annex XII - Benefit assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V Tafamidis Reassessment of an orphan drug after exceeding the € 50 million turnover limit (Amyloidosis with polyneuropathy)

of 20 May 2021

At its session on 20 May 2021, the Federal Joint Committee (G-BA) resolved to amend the Pharmaceuticals Directive, (AM-RL) in the version dated 18 December 2008/22 January 2009 (Federal Gazette, BAnz. No. 49a of 31 March 2009), as last amended on DD. Month YYYY (Federal Gazette, BAnz AT DD.MM.YYYY BX), as follows:

I. Annex XII is amended as follows:

1. The information on tafamidis in the version of the resolution of 7 June 2012 (BAnz AT 27.06.2012 B3) is repealed.

2. Annex XII shall be amended in alphabetical order to include tafamidis as follows:

Tafamidis

Resolution of: 20 May 2021
Entry into force on: 20 May 2021
BAnz AT DD. MM YYYY Bx

Therapeutic indication (according to the marketing authorisation of 16 November 2011):

Vyndaqel is indicated for the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment

Therapeutic indication of the resolution (resolution from the 20/05/2021):

see therapeutic indication according to marketing authorisation

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

Adult patients with transthyretin amyloidosis with stage 1 symptomatic polyneuropathy

Appropriate comparator therapy for tafamidis:

Patisiran

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

An additional benefit is not proven

Study results according to endpoints:[1]

Adult patients with transthyretin amyloidosis with stage 1 symptomatic polyneuropathy

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	∅	There are no usable data for the benefit assessment.
Morbidity	∅	There are no usable data for the benefit assessment.
Health-related quality of life	∅	There are no usable data for the benefit assessment.
Side effects	∅	There are no usable data for the benefit assessment.
Explanations:		

[1] Data from the dossier assessment of the Institute for Quality and Efficiency in Health Care (IQWiG) (A20-101) unless otherwise indicated.

↑: 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

No suitable data submitted.

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

Adult patients with transthyretin amyloidosis with stage 1 symptomatic polyneuropathy

approx. 230 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 Vyndaqel (active ingredient: tafamidis) at the following publicly accessible link (last access: 1 March 2021):
https://www.ema.europa.eu/documents/product-information/vyndaqel-epar-product-information_de.pdf

Treatment with tafamidis should only be initiated and monitored by doctors experienced in treating patients with amyloidosis or polyneuropathy.

This medicinal product was approved under "exceptional circumstances". This means that due to the rarity of the disease, it was not possible to obtain complete information on this medicinal product. The EMA will assess any new information that becomes available on an annual basis, and, if necessary, the summary of product characteristics will be updated.

4. Treatment costs

Annual treatment costs:

Adult patients with transthyretin amyloidosis with stage 1 symptomatic polyneuropathy

Designation of the therapy	Annual treatment costs/patient
Medicinal product to be assessed:	
Tafamidis	€ 162,590.95
Appropriate comparator therapy:	
Patisiran	€ 435,300.50
additionally required SHI services	€ 124.65

Costs after deduction of statutory rebates (LAUER-TAXE®, as last revised: 01 May 2021)

II. The resolution will enter into force on the day of its publication on the internet on the website of the G-BA on 20 May 2021.

The justification for this resolution will be published on the website of the G-BA at www.g-ba.de.

Berlin, 20 May 2021

Federal Joint Committee in accordance with Section 91 SGB V The chairman

Prof. Hecken