Ivacaftor/ Tezacaftor/ Elexacaftor (new therapeutic indication: Cystic Fibrosis, combination regimen with ivacaftor, 6 to 11 years (heterozygous for F508del and RF mutations))

Resolution of: 4 August 2022 Valid until: unlimited
Entry into force on: 4 August 2022
Federal Gazette, BAnz AT 23 09 2022 B2

New therapeutic indication (according to the marketing authorisation of 7 January 2022):

Kaftrio is indicated in a combination regimen with ivacaftor for the treatment of cystic fibrosis (CF) in patients aged 6 years and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

Therapeutic indication of the resolution (resolution of 4 August 2022):

Kaftrio is indicated in a combination regimen with ivacaftor for the treatment of cystic fibrosis in patients aged 6 to 11 years who are heterozygous for an F508del mutation in the CFTR gene and carry a residual function mutation on the second allele.

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

Children aged 6 to 11 years with cystic fibrosis who are heterozygous for the F508del mutation in the CFTR gene and carry a residual function mutation on the second allele

Appropriate comparator therapy for Ivacaftor/ Tezacaftor/ Elexacaftor in combination with Ivacaftor:

tezacaftor/ ivacaftor in combination with ivacaftor

Extent and probability of the additional benefit of Ivacaftor/ Tezacaftor/ Elexacaftor in combination with Ivacaftor compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:[1]

Children aged 6 to 11 years with cystic fibrosis who are heterozygous for the F508del mutation in the CFTR gene and carry a residual function mutation on the second allele

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ risk of bias	Summary
Mortality	∅	No data available.
Morbidity	∅	No data available.
Health-related quality of life	∅	No data available.
Side effects	∅	No data available.

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

Children aged 6 to 11 years with cystic fibrosis who are heterozygous for the F508del mutation in the CFTR gene and carry a residual function mutation on the second allele

approx. 21 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 Kaftrio (active ingredient: ivacaftor/ tezacaftor/ elexacaftor) at the following publicly accessible link (last access: 11 May 2022):

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

[1] Data from the dossier assessment of the IQWiG (A22-18, A22-24) unless otherwise indicated.

Treatment with ivacaftor/ tezacaftor/ elexacaftor should only be initiated and monitored by doctors experienced in treating children with cystic fibrosis.

4. Treatment costs

Annual treatment costs:

Children aged 6 to 11 years with cystic fibrosis who are heterozygous for the F508del mutation in the CFTR gene and carry a residual function mutation on the second allele

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Ivacaftor/ tezacaftor/ elexacaftor	€ 156,562.19
+ ivacaftor	€ 82,914.18 - € 82,970.63
Total:	€ 239,476.37 - € 239,532.81
Appropriate comparator therapy:	
Tezacaftor/ elexacaftor	€ 65,035.44
+ ivacaftor	€ 82,914.18 - € 82,970.63
Total:	€ 147,949.62 - € 148,006.07

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 July 2022)

Costs for additionally required SHI services: not applicable