Gemeinsamer Bundesausschuss

Ivacaftor (new therapeutic indication: cystic fibrosis, combination regimen with Ivacaftor/ Tezacaftor/ Elexacaftor, 6 to 11 years (heterozygous for F508del and other or unknown mutations))

Resolution of: 4 August 2022 Valid until: unlimited
Entry into force on: 4 August 2022
Federal Gazette, BAnz AT 24 08 2022 B3

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

Kalydeco tablets are indicated in a combination regimen with ivacaftor/ tezacaftor/ elexacaftor tablets for the treatment of adults, adolescents and children aged 6 years and older with cystic fibrosis (CF) who have at least one F508del mutation in the CFTR gene.

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

Kalydeco tablets are indicated in a combination regimen with ivacaftor/ tezacaftor/ elexacaftor tablets for the treatment of children aged 6 to 11 years with cystic fibrosis, who are heterozygous for the F508del mutation in the CFTR gene and carry a mutation on the second allele that is not a minimal function, gating (including R117H) or residual function mutation, or the mutation on the second allele is unknown (other mutations).

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 mutation on the second allele, which is not a minimal function, no gating (including R117H) and no residual function mutation, or the mutation on the second allele is unknown (other mutations)

Appropriate comparator therapy for ivacaftor in combination with ivacaftor/ tezacaftor/ elexacaftor:

Best supportive care

Best Supportive Care (BSC) is defined as the therapy that ensures the best possible, patient-individual optimised, supportive treatment to alleviate symptoms and improve the quality of life (in particular antibiotics for pulmonary infections, mucolytics, pancreatic enzymes for pancreatic insufficiency, physiotherapy (as defined in the Remedies Directive), making full use of all possible dietary measures).

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

An additional benefit is not proven.

Study results according to endpoints:

Children aged 6 to 11 years with cystic fibrosis, who are heterozygous for the F508del mutation in the CFTR gene and carry a mutation on the second allele, which is not a minimal function, no gating (including R117H) and no residual function mutation, or the mutation on the second allele is unknown (other mutations)

No 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	∅	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 mutation on the second allele, which is not a minimal function, no gating (including R117H) and no residual function mutation, or the mutation on the second allele is unknown (other mutations)

approx. 56 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 Kalydeco (active ingredient: ivacaftor) at the following publicly accessible link (last access: 17 May 2022):

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

Treatment with ivacaftor should only be initiated and monitored by doctors experienced in the therapy of 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 show a mutation on the second allele, which is not a minimal function, no gating (including R117H) and no residual function mutation, or the mutation on the second allele is unknown (other mutations)

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Ivacaftor	€ 82,914.18 - € 82,970.63
+ ivacaftor/ tezacaftor/ elexacaftor	€ 156,562.19
Total:	€ 239,476.37 - € 239,532.81
+ best supportive care	Different from patient to patient
Appropriate comparator therapy:	
Best supportive care	Different from patient to patient

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

Costs for additionally required SHI services: not applicable