Gemeinsamer Bundesausschuss

Ivacaftor (new therapeutic indication: cystic fibrosis, combination therapy with tezacaftor/ivacaftor in patients aged 6 < 12 years (homozygous or F508del-Mutation))

Resolution of: 20 May 2021 Valid until: unlimited
Entry into force on: 20 May 2021
BAnz AT 17 06 2021 B5

New therapeutic indication (according to the marketing authorisation of 25 November 2020):

Kalydeco tablets are used in combination with tezacaftor/ivacaftor tablets to treat adults, adolescents, and children 6 years of age and older with cystic fibrosis (CF) who are homozygous for the F508del-Mutation or heterozygous for the F508del-Mutation and have one of the following mutations in the CFTR gene: P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G and 3849+10kbC→T.

Therapeutic indication of the resolution (resolution of 20 May 2021):

Kalydeco tablets are used as part of a combination treatment with tezacaftor/ivacaftor tablets for the treatment of children aged 6 years < 12 years with cystic fibrosis (CF) who are homozygous for the F508del mutation.

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

Children with cystic fibrosis aged 6 < 12 years who are homozygous for the F508del mutation.

Appropriate comparator therapy:

Lumacaftor/Ivacaftor

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

An additional benefit is not proven.

Study results according to endpoints:

Children with cystic fibrosis aged 6 < 12 years who are homozygous for the F508del mutation.

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

Courtesy translation – only the German version is legally binding.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	n.c.	There are no evaluable data.
Morbidity	n.c.	There are no evaluable data.
Health-related quality of life	n.c.	There are no evaluable data.
Side effects	n.c.	There are no evaluable 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 is no usable data for the benefit assessment.
n.a.: not assessable

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

Children with cystic fibrosis aged 6 < 12 years who are homozygous for the F508del mutation.

approx. 470 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: 1 April 2021):
https://www.ema.europa.eu/en/documents/product-information/kalydeco-epar-product-information_de.pdf

Treatment with ivacaftor should only be initiated and monitored by doctors experienced in treating patients with cystic fibrosis.

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4. Treatment costs

Annual treatment costs:

Name of therapy	Annual treatment costs/patient
Medicinal product to be assessed:	
Ivacaftor[1]	€ 100,977.84 – € 101,032.65
+ Tezacaftor/ Ivacaftor	€ 65,032.44
Total:	€ 166,010.28 – € 166,065.09
Appropriate comparator therapy:	
Lumacaftor/Ivacaftor	€ 148,415.91

Costs after deduction of statutory rebates (LAUER-TAXE®, as last revised: 1 May 2021)

Costs for additionally required SHI services: not applicable

[1] The range of ivacaftor is based on different doses depending on body weight (<30 kg bw or ≥30 kg bw, respectively)

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