Gemeinsamer Bundesausschuss

Ivacaftor (new therapeutic indication: cystic fibrosis, patients 4 < 6 months of age, R117H-Mutation)

Resolution of: 20 May 2021 Valid until: unlimited
Entry into force on: 20 May 2021
BAnz AT 22 06 2021 B3

New therapeutic indication (according to the marketing authorisation of 3 November 2020):

Kalydeco granules are used to treat infants 4 months of age and older, toddlers and children weighing between 5 kg and less than 25 kg with cystic fibrosis (CF, mucoviscidosis) who have an R117H CFTR-Mutation or one of the following gating mutations (class III) in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R.

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

Kalydeco granules are used to treat infants with cystic fibrosis aged 4 < 6 months who have an R117H-Mutation in the CFTR gene.

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

Infants with cystic fibrosis aged 4 to < 6 months who have an R117H-Mutation in the CFTR gene

Appropriate comparator therapy for ivacaftor:

– Best supportive care

Best Supportive Care (BSC) is understood to be the therapy that ensures the best possible, patient-individually 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), with exhaustion of all possible dietary measures).

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

Indication of non-quantifiable additional benefit

Study results according to endpoints:[1]

Infants with cystic fibrosis aged 4 to < 6 months who have an R117H-Mutation in the CFTR gene

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	↔	No relevant difference for the benefit assessment Differences under transfer of evidence Outcomes of patients ≥ 18 years with R117H-Mutation
Morbidity	↑	Advantages under transfer of evidence Outcomes of patients ≥ 18 years with R117H-Mutation
Health-related quality of life	↑	Advantages under transfer of evidence Outcomes of patients ≥ 18 years with R117H-Mutation
Side effects	↔	No relevant difference for the benefit assessment Differences taking into account the results from patients 4 < 6 months with gating mutations
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		

Study results

There are no usable data for the benefit assessment.

[1] Data from the dossier assessment of the Institute for Quality and Efficiency in Health Care (IQWiG) (A20-99) unless otherwise indicated.

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

Infants with cystic fibrosis aged 4 to < 6 months who have an R117H-Mutation in the CFTR gene

approx. 1 patient

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: 06 May 2021):
https://www.ema.europa.eu/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.

4. Treatment costs

Annual treatment costs:

Infants with cystic fibrosis aged 4 to < 6 months who have an R117H-Mutation in the CFTR gene

Name of therapy	Annual treatment costs/patient
Medicinal product to be assessed:	
Ivacaftor	€ 201,955.67
Best supportive care	Patient-individual
Appropriate comparator therapy:	
Best supportive care	Patient-individual

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

Costs for additionally required SHI services: not applicable