Gemeinsamer Bundesausschuss

# Resolution

of the Federal Joint Committee on an Amendment of the Pharmaceuticals Directive:
Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V
Ivacaftor (new therapeutic indication: cystic fibrosis, combination regimen with Ivacaftor/ Tezacaftor/ Elexacaftor, 6 to 11 years (heterozygous for F508del and gating mutations (including R117H))

of 4 August 2022

At its session on 4 August 2022, 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 by the publication of the resolution of D Month YYYY (Federal Gazette, BAnz AT DD.MM.YYYY BX), as follows:

**I. In Annex XII, the following information shall be added after No. 4 to the information on the benefit assessment of ivacaftor in accordance with the resolution of 19.11.2021:**

**Ivacaftor**

Resolution of: 4 August 2022
Entry into force on: 4 August 2022
Federal Gazette, BAnz AT DD. MM YYYY Bx

**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 cystic fibrosis in patients aged 6 to 11 years who are heterozygous for the F508del mutation in the CFTR gene and carry a gating mutation (including R117H) 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 gating mutation (including R117H) on the second allele

**Appropriate comparator therapy:**

Ivacaftor

**Extent and probability of the additional benefit of ivacaftor in combination with Ivacaftor/ Tezacaftor/ Elexacaftor:**

An additional benefit is not proven.

**Study results according to endpoints:**[1]

No suitable data versus the appropriate comparator therapy available.

[1] Data from the dossier assessment of the IQWiG (A22-17 and A22-23) unless otherwise indicated.

**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 gating mutation (including R117H) on the second allele

approx. 28 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: 13 July 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 carry a gating mutation (including R117H) on the second allele

| 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 |
| Appropriate comparator therapy: | |
| Ivacaftor | € 165,828.36 |

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

Costs for additionally required SHI services: not applicable

**II. The resolution will enter into force on the day of its publication on the website of the G-BA on 4 August 2022.**

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

Berlin, 4 August 2022

Federal Joint Committee (G-BA)
in accordance with Section 91 SGB V
The Chair

Prof. Hecken