

Tezacaftor/ivacaftor (new therapeutic indication: cystic fibrosis, combination therapy with 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

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

Symkevi is used as a combination treatment with ivacaftor tablets for the treatment of cystic fibrosis (CF) in patients 6 years of age and older who are homozygous for the F508del-Mutation or heterozygous for the F508del-Mutation and have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (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):

Symkevi is used as a combination treatment with ivacaftor tablets for the treatment of cystic fibrosis (CF) in children 6 years < 12 years of age 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 tezacaftor/ivacaftor in combination with 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.

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 Symkevi (active ingredient: tezacaftor/ivacaftor) at the following publicly accessible link (last access: 1 April 2021):

https://www.ema.europa.eu/en/documents/product-information/symkevi-epar-product-information_de.pdf

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

4. Treatment costs

Annual treatment costs:

Name of therapy	Annual treatment costs/patient
Medicinal product to be assessed:	
Tezacaftor/ivacaftor	€ 65,032.44
+ Ivacaftor ¹	€ 100,977.84 – € 101,032.65
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)