Riociguat (Reassessment of an Orphan Drug after Exceeding the € 50 Million Limit: CTEPH)

Resolution of: 3 September 2020 valid until: unlimited
Entry into force on: 3 September 2020
Federal Gazette, BAnz AT 25 09 2020 B4

Therapeutic indication (according to the marketing authorisation of 27 March 2014):

Adempas is indicated for the treatment of adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment, to improve exercise capacity.

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

Adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment

Appropriate comparator therapy:

-best supportive care

Best supportive care (BSC) is the therapy that ensures the best possible, patient-individual, supportive treatment to alleviate symptoms and improve the quality of life.

Extent and probability of the additional benefit of riociguat compared with the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:

Adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment

No suitable data were submitted for the benefit assessment.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	n.a.	No data suitable for the benefit assessment
Morbidity	n.a.	No data suitable for the benefit assessment
Health-related quality of life	n.a.	No data suitable for the benefit assessment
Side effects	n.a.	No data suitable for the benefit assessment
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

Adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment

approx. 920 to 5,460 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 Adempas® (active ingredient: riociguat) at the following publicly accessible link (last access: 18 June 2020):

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

Treatment with riociguat should only be initiated and monitored by specialists who are experienced in the treatment of patients with CTEPH.

4. Treatment costs

Annual treatment costs:

Adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment

Designation of the therapy	Annual treatment costs/patient
Medicinal product to be assessed:	
Riociguat	€ 32,673.87 – 33,178.11
Best supportive care	different for each individual patient
Appropriate comparator therapy:	
Best supportive care	different for each individual patient

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 August 2020

Costs for additionally required SHI services: not applicable