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
Ravulizumab (new therapeutic indication: paroxysmal haemoglobinuria, paediatric patients)

of 18 March 2022

At its session on 18 March 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 Ravulizumab in accordance with the resolution of 21 January 2021:

Ravulizumab

Resolution of: 18 March 2022
Entry into force on: 18 March 2022
Federal Gazette, BAnz AT DD. MM YYYY Bx

New therapeutic indication (according to the marketing authorisation of 1 September 2021):

Ultomiris is indicated in the treatment of adult and paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH):

- in patients with haemolysis with clinical symptom(s) indicative of high disease activity,
- in patients who are clinically stable after having been treated with eculizumab for at least the past 6 months.

Therapeutic indication of the resolution (resolution of 18 March 2022):

Ultomiris is indicated in the treatment of paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH):

- in patients with haemolysis with clinical symptom(s) indicative of high disease activity,
- in patients who are clinically stable after having been treated with eculizumab for at least the past 6 months.

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

a) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) and haemolysis with clinical symptom(s) indicative of high disease activity

Appropriate comparator therapy:

- Eculizumab

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

An additional benefit is not proven.

b) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) who are clinically stable after having been treated with eculizumab for at least the past 6 months

Appropriate comparator therapy:

- Eculizumab

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

An additional benefit is not proven.

Study results according to endpoints:

a) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) and haemolysis with clinical symptom(s) indicative of high disease activity

No 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.a.	There are no assessable data.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	∅	No data available.
Side effects	n.a.	There are no assessable 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 are no usable data for the benefit assessment. n.a.: not assessable		

b) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) who are clinically stable after having been treated with eculizumab for at least the past 6 months

No 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.a.	There are no assessable data.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	∅	No data available.
Side effects	n.a.	There are no assessable 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 are no usable data for the benefit assessment. n.a.: not assessable

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

a) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) and haemolysis with clinical symptom(s) indicative of high disease activity

approx. 0 - 35 patients

b) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) who are clinically stable after having been treated with eculizumab for at least the past 6 months

approx. 0 - 14 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 Ultomiris (active ingredient: ravulizumab) at the following publicly accessible link (last access: 9 February 2022):

https://www.ema.europa.eu/en/documents/product-information/ultomiris-epar-product-information_en.pdf

Treatment with ravulizumab should only be initiated and monitored by specialists who are experienced in the treatment of patients with haematological diseases.

In accordance with the European Medicines Agency (EMA) requirements regarding additional risk minimisation measures, the pharmaceutical company must provide training material that contains information for medical professionals and patients. In particular, the training material contains instructions regarding the increased risk of meningococcal infection under ravulizumab.

4. Treatment costs

Annual treatment costs:

a) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) and haemolysis with clinical symptom(s) indicative of high disease activity

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Ravulizumab	€ 136,872.06 - € 375,947.91
Appropriate comparator therapy:	
Eculizumab	€ 144,619.84 - € 433,859.52

Costs after deduction of statutory rebates (LAUER-TAXE® as last revised: 1 March 2022)

Costs for additionally required SHI services: not applicable

Other SHI services:

Designation of the therapy	Type of service	Costs/ unit	Number/ cycle	Number/ patient/ year	Costs/ patient/ year
Medicinal product to be assessed:					
Ravulizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 71	1	6.5	€ 461.50
				13.0	€ 923.00
Appropriate comparator therapy:					
Eculizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 71	1	26.1	€ 1,853.10

b) Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH) who are clinically stable after having been treated with eculizumab for at least the past 6 months

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Ravulizumab	€ 136,872.06 - € 375,947.91

Designation of the therapy	Annual treatment costs/ patient
Appropriate comparator therapy:	
Eculizumab	€ 144,619.84 - € 433,859.52

Costs after deduction of statutory rebates (LAUER-TAXE® as last revised: 1 March 2022)

Costs for additionally required SHI services: not applicable

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Medicinal product to be assessed:					
Ravulizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 71	1	6.5	€ 461.50
				13.0	€ 923.00
Appropriate comparator therapy:					
Eculizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 71	1	26.1	€ 1,853.10

II. The resolution will enter into force on the day of its publication on the website of the G-BA on 18 March 2022.

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

Berlin, 18 March 2022

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

Prof. Hecken