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

Iptacopan (reassessment of orphan drug > 30 million: paroxysmal nocturnal haemoglobinuria)

From 4 June 2026

At their session on 4 June 2026, 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 information on the active ingredient Iptacopan in the version of the resolution of 19 December 2024 (Federal Gazette, BAnz AT 13.02.2025 B1) shall be replaced by the following information:

Iptacopan

Resolution of: 4 June 2026
Entry into force on: 4 June 2026
Federal Gazette, BAnz AT DD. MM YYYY Bx

Therapeutic indication (according to the marketing authorisation of 17 May 2024):

Fabhalta is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.

Therapeutic indication of the resolution (resolution of 4 June 2026):

See therapeutic indication according to marketing authorisation.

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

a) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not been pretreated

Appropriate comparator therapy:

- eculizumab

or

- ravulizumab

or

- pegcetacoplan

Extent and probability of the additional benefit of iptacopan as monotherapy compared to the appropriate comparator therapy:

An additional benefit is not proven.

b) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia and have been pretreated

Appropriate comparator therapy:

- pegcetacoplan

or

- eculizumab + danicopan

or

- ravulizumab + danicopan

Extent and probability of the additional benefit of iptacopan as monotherapy compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:[1]

a) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not been pretreated

No data available.

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 ∅: No data available. n.a.: not assessable		

b) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia and have been pretreated

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	n.a.	There are no assessable data.
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 ∅: No data available. n.a.: not assessable		

[1] Data from the dossier assessment of the IQWiG (A25-156) and from the addendum (A26-43), unless otherwise indicated.

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

a) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not been pretreated

Approx. 100 - 425 patients

b) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia and have been pretreated

Approx. 190 - 520 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 Fabhalta (active ingredient: iptacopan) at the following publicly accessible link (last access: 13 April 2026):

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

Treatment with iptacopan 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 (incl. patient card). The training material contains, in particular, informations and warnings of the increased risk of infection with encapsulated bacteria associated with the use of iptacopan.

4. Treatment costs

Annual treatment costs:

a) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not been pretreated

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Iptacopan	€ 383,152.15
Appropriate comparator therapy:	
eculizumab or ravulizumab or pegcetacoplan	
eculizumab	€ 360,229.28 - € 480,305.71
ravulizumab	€ 301,859.03
pegcetacoplan	€ 362,401.56

Costs after deduction of statutory rebates (LAUER-TAXE® as last revised: 1 April 2026)

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
eculizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	22.8 – 30.4	€ 2,280 - € 3,040
ravulizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	6.5	€ 650

b) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia and have been pretreated

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Iptacopan	€ 383,152.15
Appropriate comparator therapy:	
pegcetacoplan	
pegcetacoplan	€ 362,401.56
eculizumab + danicopan	
eculizumab	€ 360,229.28 - € 480,305.71
danicopan	€ 86,261.30 - € 114,979.75
Total:	€ 446,490.58 - € 595,285.46
ravulizumab + danicopan	
ravulizumab	€ 301,859.03
danicopan	€ 86,261.30 - € 114,979.75
Total:	€ 388,120.33 - € 416,838.78

Costs after deduction of statutory rebates (LAUER-TAXE® as last revised: 1 April 2026)

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
eculizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	22.8 – 30.4	€ 2,280 - € 3,040
ravulizumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	6.5	€ 650

5. Designation of medicinal products with new active ingredients according to Section 35a, paragraph 3, sentence 4 SGB V that can be used in a combination therapy with the assessed medicinal product

In the context of the designation of medicinal products with new active ingredients pursuant to Section 35a, paragraph 3, sentence 4 SGB V, the following findings are made:

a) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not been pretreated

- No designation of medicinal products with new active ingredients that can be used in combination therapy pursuant to Section 35a, paragraph 3, sentence 4 SGB V, as the active ingredient to be assessed is an active ingredient approved as monotherapy.

b) Adults with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia and have been pretreated

- No designation of medicinal products with new active ingredients that can be used in combination therapy pursuant to Section 35a, paragraph 3, sentence 4 SGB V, as the active ingredient to be assessed is an active ingredient approved as monotherapy.

The designation of combinations exclusively serves the implementation of the combination discount according to Section 130e SGB V between health insurance funds and pharmaceutical companies. The findings made neither restrict the scope of treatment required to fulfil the medical treatment mandate, nor do they make statements about expediency or economic feasibility.

II. The resolution will enter into force on the day of its publication on the G-BA website on 4 June 2026.

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

Berlin, 4 June 2026

Federal Joint Committee
in accordance with Section 91 SGB V
The Chair

Prof. Hecken