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 an orphan drug after exceeding the EUR 30 million limit: complement 3 glomerulopathy)

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 following information shall be added after No. 5 to the information on the benefit assessment of Iptacopan in accordance with the resolution of 4 June 2026:

Iptacopan

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

New therapeutic indication (according to the marketing authorisation of 31 March 2025):

FABHALTA is indicated for the treatment of adult patients with complement 3 glomerulopathy (C3G) in combination with a renin-angiotensin system (RAS) inhibitor, or in patients who are RAS-inhibitor intolerant, or for whom a RAS inhibitor is contraindicated

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

See new therapeutic indication according to marketing authorisation.

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

Adults with complement 3 glomerulopathy (C3G)

Appropriate comparator therapy:

- Mycophenolate mofetil in combination with corticosteroids

Extent and probability of the additional benefit of iptacopan, alone or in combination with renin-angiotensin system (RAS) inhibitors compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:[1]

Adults with complement 3 glomerulopathy (C3G)

[1] Data from the dossier assessment by the IQWiG (A25-157), unless otherwise indicated.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ risk of bias	Summary
Mortality	↔	No deaths occurred.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	n.a.	There are no assessable data.
Side effects	↔	No relevant difference 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
∅: No data available.
n.a.: not assessable

APPEAR-C3G study: randomised controlled trial, iptacopan vs placebo (each in combination with mycophenolate mofetil and corticosteroids), 6-month comparator study duration

Mortality

Endpoint	Iptacopan + MMF + GC		Placebo + MMF + GC		Iptacopan + MMF + GC vs Placebo + MMF + GC
	N	Patients with event n (%)	N	Patients with event n (%)	RR [95% CI] p value[a]
Overall mortality[b]	9	0 (0)	9	0 (0)	–

Morbidity

Endpoint	Iptacopan + MMF + GC		Placebo + MMF + GC		Iptacopan + MMF + GC vs Placebo + MMF + GC
	N	Patients with event n (%)	N	Patients with event n (%)	RR [95% CI] p value[a]
Fatigue (FACIT-Fatigue, PGI-S)	No suitable data[c]				
Health status (EQ-5D VAS)	No suitable data[c]				

	Iptacopan + MMF + GC	Placebo + MMF + GC	Iptacopan + MMF + GC vs Placebo + MMF + GC
			Mean difference [95% CI] p value
Proteinuria – change from baseline in the UPCR at month 6 (FAS) (presented additionally)[e]			
N *Baseline mean value (SD)*	*9* *1.0 (0.8)*	*9* *1.0 (0.5)*	
N *Day 180 mean value (SD)* *Day 180: adjusted mean change (SE)*	*9* *1.2 (0.6)* *0.2 (0.1)*	*9* *1.2 (0.5)* *0.2 (0.1)*	*-0.0 [-0.38; 0.37] 0.979*
N *Overall treatment effect: LS mean (SE)*	*9* *-0.2 (0.2)*	*9* *0.2 (0.2)*	*-0.4 [-0.89; 0.16] 0.159*

Health-related quality of life

Endpoint	Iptacopan + MMF + GC		Placebo + MMF + GC		Iptacopan + MMF + GC vs Placebo + MMF + GC
	N	Patients with event n (%)	N	Patients with event n (%)	RR [95% CI] p value[a]
SF-36					
Physical Component Summary (PCS) score	No suitable data[c]				
Mental Component Summary (MCS) score	No suitable data[c]				

Side effects

Endpoint	Iptacopan + MMF + GC		Placebo + MMF + GC		Iptacopan + MMF + GC vs Placebo + MMF + GC
	N	Patients with event n (%)	N	Patients with event n (%)	RR [95% CI] p value[a]
AEs (presented additionally)	9	9 (100)	9	8 (88.9)	–
SAEs	9	1 (11.1)	9	1 (11.1)	1.00 [0.07; 13.64]; > 0.999
Discontinuation due to AEs	9	0 (0)	9	0 (0)	–
Infections[d] (AEs)	9	4 (44.4)	9	4 (44.4)	1.00 [0.36; 2.81]; > 0.999
Infections[d] (SAEs)	9	0 (0)	9	0 (0)	–

a. IQWiG calculation of RR, CI (asymptotic) and p value (unconditional exact test, CSZ method).
b. The results on overall mortality are based on the data on fatal AEs.
c. Significant difference (> 15 percentage points) between the percentages of patients not included in the analysis (for the reasoning, see Justification)
d. Operationalised as infections and infestations (SOC)
e. Data from the dossier of the pharmaceutical company

Abbreviations used:
FACIT = Functional Assessment of Chronic Illness Therapy; FAS = Full Analysis Set; GC = glucocorticoids; CI = confidence interval; LS = least squares; MMF = mycophenolate mofetil; MV = mean value; n = number of patients with (at least 1) event; N = number of patients evaluated; RCT = randomised controlled trial; RR = relative risk; SF-36 = Short Form-36 Health Survey; SD = standard deviation; SE = standard error; SOC = system organ class; SAE = serious adverse event; AE = adverse event; VAS = visual analogue scale

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

Adults with complement 3 glomerulopathy (C3G)

Approx. 110 – 230 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: 25 March 2026):

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

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:

The costs for the first year of treatment are shown for the cost representation in the resolution.

Adults with complement 3 glomerulopathy (C3G)

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Iptacopan	€ 383,152.15

Designation of the therapy	Annual treatment costs/ patient
Appropriate comparator therapy:	
Mycophenolate mofetil in combination with corticosteroids	
Mycophenolate mofetil	€ 1,099.79 - € 2,199.58
Prednisone	€ 26.54 - € 53.07
Total:	€ 1,126.33 - € 2,252.65

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

Costs for additionally required SHI services: not applicable

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:

Adults with complement 3 glomerulopathy (C3G)

- No medicinal product with new active ingredients for use in combination therapy in compliance with the requirements of Section 35a, paragraph 3, sentence 4 SGB V.

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