Drug assessment at a glance: A25-156[1]

Iptacopan (paroxysmal nocturnal haemoglobinuria)

20 July 2026

[1] This is a courtesy translation of the summary provided as a service by IQWiG. For further information on the subject matter and the result of the assessment, please refer to the dossier assessment for Project A25-156. The G-BA decides on the added benefit.

Research question 1 of the benefit assessment

Population	Adults with paroxysmal nocturnal haemoglobinuria (PNH) with haemolytic anaemia who are treatment-naive
Intervention	iptacopan
Comparator	▪ eculizumab or ▪ ravulizumab
Outcomes	At least one patient-relevant outcome

Overview of positive and negative effects – research question 1

	Size of the effects	Informative value of the effects
Mortality	∅	–
Morbidity	∅	–
Symptoms	∅	–
Health-related quality of life	∅	–
Side effects	∅	–
	∅ No (suitable) data ↔ No advantage or disadvantage ↑ / ↓ Small advantage/disadvantage ↑↑ / ↓↓ Moderate advantage/disadvantage ↑↑↑ / ↓↓↓ Large advantage/disadvantage	– Not applicable ★ Low ★★ Moderate ★★★ High

Overall: No advantage or disadvantage (no suitable data)

Evidence base and noteworthy aspects – research question 1

- No data were available.

Research question 2 of the benefit assessment

Population	Adults with PNH who still have haemolytic anaemia and have received prior treatment
Intervention	iptacopan
Comparator	▪ pegcetacoplan or ▪ eculizumab + danicopan or ▪ ravulizumab + danicopan
Outcomes	At least one patient-relevant outcome

Overview of positive and negative effects – research question 2

	Size of the effects	Informative value of the effects
Mortality	∅	–
Morbidity	∅	–
Symptoms	∅	–
Health-related quality of life	∅	–
Side effects	∅	–
	∅ No (suitable) data ↔ No advantage or disadvantage ↑ / ↓ Small advantage/disadvantage ↑↑ / ↓↓ Moderate advantage/disadvantage ↑↑↑ / ↓↓↓ Large advantage/disadvantage	– Not applicable ★ Low ★★ Moderate ★★★ High

Overall: No advantage or disadvantage (no suitable data)

Evidence base and noteworthy aspects – research question 2

- The APPLY-PNH study (an open-label RCT comparing iptacopan versus continued treatment with eculizumab or ravulizumab) was not suitable, as it did not allow for a comparison of iptacopan with the comparator therapy.

References

Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen. Iptacopan (paroxysmale nächtliche Hämoglobinurie); Nutzenbewertung gemäß § 35a SGB V; Dossierbewertung [online]. 2026 [Accessed: 04.06.2026]. URL: https://doi.org/10.60584/A25-156.

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English translations for this project are published at https://www.iqwig.de/en/projects/a25-156.html and https://www.iqwig.de/en/projects/a26-43.html.

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