Drug assessment at a glance: A25-136[1]

Avapritinib (indolent systemic mastocytosis)

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 A25-136. The G-BA decides on the added benefit.

Research question for the benefit assessment

Population	Adult patients with indolent systemic mastocytosis (ISM) with moderate to severe symptoms inadequately controlled on symptomatic treatment
Intervention	avapritinib
Comparator	Individualized treatment from a choice of ▪ cladribine ▪ peginterferon alfa-2a ± prednisone ▪ midostaurin ▪ best supportive care
Outcomes	At least one patient-relevant outcome

Overview of positive and negative effects

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

Overall: No advantage or disadvantage (no suitable data)

Evidence base and noteworthy aspects

- Evidence base: double-blind, randomized PIONEER study comparing avapritinib + BSC (n = 141) with placebo + BSC (n = 71)
- Individualized treatment decisions were not possible in the study. It is unclear whether in PIONEER the best possible, individually optimized treatment to alleviate symptoms was provided. The study was therefore not suitable for answering the research question.

References

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English translations for this project are published at https://www.iqwig.de/en/projects/a25-136.html and https://www.iqwig.de/en/projects/a26-28.html.

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