

Drug assessment at a glance: A25-147¹

Selumetinib (plexiform neurofibromas in adults with neurofibromatosis type 1)

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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 A25-147. The G-BA decides on the added benefit.

Research question for the benefit assessment

Population	Adult patients with symptomatic, inoperable plexiform neurofibromas in neurofibromatosis type 1
Intervention	selumetinib
Comparator	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 (chronic pain, spikes of pain)	↑		★	
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: a small advantage with low informative value

Evidence base and noteworthy aspects

- Evidence base: KOMET study (double-blind RCT; selumetinib [N = 71] versus placebo [N = 74])
- There was a high risk of bias regarding morbidity, health status and side effects (except for discontinuation due to AEs) due to incomplete observations.
- No suitable data were available regarding physical functioning.
- Health-related quality of life was not recorded.

Key study findings

Study Outcome category Outcome	selumetinib		placebo		selumetinib vs. placebo
	N	Patients with event n (%)	N	Patients with event n (%)	RR [95% CI]; p-value
KOMET					
Morbidity					
Improvement by ≥ 15 % of the scale range at Week 48					
Chronic pain (NRS)	57	25 (43.9)	62	16 (25.8)	1.70 [1.02; 2.84]; 0.042
Spikes of pain (NRS)	58	39 (67.2)	64	25 (39.1)	1.72 [1.21; 2.45]; 0.002
Side effects					
SAEs	71	10 (14.1)	74	9 (12.2)	1.16 [0.50; 2.68]; 0.775
Severe AEs	71	23 (32.4)	74	13 (17.6)	1.84 [1.01; 3.35]; 0.043
Discontinuation due to AEs	71	9 (12.7)	74	5 (6.8)	1.88 [0.66; 5.33]; 0.254

References

Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen. Selumetinib (plexiforme Neurofibrome bei Neurofibromatose Typ 1, Erwachsene); Nutzenbewertung gemäß § 35a SGB V; Dossierbewertung [online]. 2026 [Accessed: 11.05.2026]. URL: <https://doi.org/10.60584/A25-147>.

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English translations for this project are published at <https://www.iqwig.de/en/projects/a25-147.html> and <https://www.iqwig.de/en/projects/a26-33.html>.

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