

Drug assessment at a glance: A25-146<sup>1</sup>

## **Selumetinib (plexiform neurofibromas in neurofibromatosis type 1, $\geq 3$ to $< 18$ years)**

20 July 2026

---

<sup>1</sup> 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 A25-146. The G-BA decides on the added benefit.

# Research question for the benefit assessment

|              |                                                                                                                             |
|--------------|-----------------------------------------------------------------------------------------------------------------------------|
| Population   | Paediatric patients aged 3 years and older 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 |                |
|---------------------------------------|---------------------|---------------------------------|----------------------------------|----------------|
| <b>Mortality</b>                      | ∅                   |                                 | –                                |                |
| <b>Morbidity</b>                      | ∅                   |                                 | –                                |                |
| <b>Symptoms</b>                       | ∅                   |                                 | –                                |                |
| <b>Health-related quality of life</b> | ∅                   |                                 | –                                |                |
| <b>Side effects</b>                   | ∅                   |                                 | –                                |                |
|                                       | ∅                   | 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

- No suitable data were available. The dossier presented 6 single-arm studies with selumetinib and 5 studies on the natural course of the disease. The studies were not suitable for the benefit assessment, particularly due to the lack of comparative evidence.

## References

Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen. Selumetinib (plexiforme Neurofibrome bei Neurofibromatose Typ 1,  $\geq 3$  bis  $< 18$  Jahre); Nutzenbewertung gemäß § 35a SGB V; Dossierbewertung [online]. 2026 [Accessed: 08.05.2026]. URL: <https://doi.org/10.60584/A25-146>.

Gemeinsamer Bundesausschuss. Nutzenbewertungsverfahren zum Wirkstoff Selumetinib (Neubewertung Orphan  $> 30$  Mio: Neurofibromatose Typ 1 ( $\geq 3$  bis  $< 18$  Jahre)) [online]. 2026 [Accessed: 08.05.2026]. URL: <https://www.g-ba.de/bewertungsverfahren/nutzenbewertung/1284/>.

English translations for this project are published at <https://www.iqwig.de/en/projects/a25-146.html>.

# Publisher details

## **Publisher**

Institute for Quality and Efficiency in Health Care (IQWiG)

## **Address**

Siegburger Str. 237

50679 Köln

Germany

Phone: +49 221 35685-0

Fax: +49 221 35685-1

Email: [info@iqwig.de](mailto:info@iqwig.de)

Internet: [www.iqwig.de](http://www.iqwig.de)