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
Selumetinib (new therapeutic indication: neurofibromatosis type 1 (≥ 1 to < 3 years))

From 6 August 2026

At their session on 6 August 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 number 5 to the information on the benefit assessment of selumetinib in accordance with the resolution of 7 May 2026 on the therapeutic indication "Neurofibromatosis type 1 (≥ 18 years)":

Selumetinib

Resolution of: 6 August 2026
Entry into force on: 6 August 2026
Federal Gazette, BAnz AT DD. MM YYYY Bx

New therapeutic indication (according to the marketing authorisation of 9 January 2026):

Koselugo as monotherapy is indicated for the treatment of symptomatic, inoperable plexiform neurofibromas (PN) in patients with neurofibromatosis type 1 (NF1) aged 1 year to less than 7 years and for older patients with swallowing difficulties.

Therapeutic indication of the resolution (resolution of 6 August 2026):

Koselugo as monotherapy is indicated for the treatment of symptomatic, inoperable plexiform neurofibromas (PN) in patients with neurofibromatosis type 1 (NF1) aged 1 year to less than 3 years.

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

Children aged 1 year to less than 3 years with symptomatic, inoperable plexiform neurofibromas (PN) associated with neurofibromatosis type 1 (NF1)

Appropriate comparator therapy for selumetinib as monotherapy:

- Best supportive care

Extent and probability of the additional benefit of selumetinib compared to best supportive care:

Hint of non-quantifiable additional benefit

Study results according to endpoints:[1]

Children aged 1 year to less than 3 years with symptomatic, inoperable plexiform neurofibromas (PN) associated with neurofibromatosis type 1 (NF1)

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ risk of bias	Summary
Mortality	n.a.	There are no assessable data.
Morbidity	↑	Advantage in the endpoint "change in tumour volume" (compared to baseline).
Health-related quality of life	n.a.	There are no assessable data.
Side effects	n.a.	There are no assessable data.
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		

SPRINKLE study: an ongoing, open-label, single-arm phase I/II study

2nd data cut-off: 16.12.2025

Study population: Children aged 1 year to less than 7 years with symptomatic, inoperable PN associated with NF1

Relevant sub-population: Children aged 1 year to less than 3 years

Mortality

No deaths occurred in the study.

Morbidity

Endpoint	Selumetinib		
	N	Volume at the start of the study in ml MV (SD)	Percentage change MV (SD)
Change in volume of the target lesion: best change in volume achieved[a]			
	13	37.84 (40.27)	–9.61 (20.86)

[1] Data from the dossier assessment of the IQWiG (A26-09) and from the addendum (A26-76), unless otherwise indicated.

Endpoint	Selumetinib	
	N	Patients with event n (%)
Objective response rate (ORR, presented additionally)[b]		
	13	2 (15.4)
Progression-free survival (PFS, presented additionally)[c]		
	13	5 (38.5)

Endpoint	Selumetinib		
	N	Number of patients at the start of the study n (%)	Number of patients in cycle 25 n (%)
Pain (GIS-pNF, parent-reported, presented additionally)[d]			
No pain	13	9 (69.2)	13 (100)
Mild	13	2 (15.4)	0 (0)
Moderate	13	2 (15.4)	0 (0)
Severe	13	0 (0)	0 (0)

Health-related quality of life

Endpoint	Selumetinib		
	N	Values at the start of the study MV (SD)	Change at cycle 25 LS MV (SE)[e]
PedsQL (parent-reported, presented additionally)[f]			
Total score	13	82.64 (14.71)	5.19 (3.48)

Side effects

Endpoint	Selumetinib	
	N	Patients with event n (%)
Total adverse events (presented additionally)		
	13	13 (100)
Serious adverse events (SAEs)		
	13	3 (23.1)
Severe adverse events (CTCAE grade ≥ 3)		
	13	6 (46.2)
Therapy discontinuation due to adverse events		
	13	0 (0)
SAE with incidence ≥ 10% **SOC**		

Endpoint	Selumetinib	
	N	Patients with event n (%)
PT		
Infections and infestations	13	3 (23.1)
AE CTCAE grade 3 or higher with incidence ≥ 10% **SOC** PT		
Infections and infestations	13	2 (15.4)

[a] Assessment by an independent centralised review based on the REiNS criteria; the target lesion was defined as the most clinically relevant plexiform neurofibroma suitable for volumetric MRI analysis. The best change achieved is the maximum reduction in volume compared with the start of the study, or the minimum increase in volume if there was no reduction in volume.
[b] Partial or complete response confirmed by an independent centralised review based on the REiNS criteria.
[c] Progression according to REiNS criteria.
[d] Parent-reported scale for the severity of pain caused by plexiform neurofibroma over the last 7 days (0: "none", 1: "mild", 2: "moderate", 3: "severe").
[e] Analysis using a mixed model for repeated measures (MMRM).
[f] PedsQL infant scale (age: 13 to 24 months) and PedsQL generic core scale (age: 2 to 4 years), both acute versions with a 7-day recall period; higher (increasing) scores indicate a better health-related quality of life (scale range 0 to 100).

Abbreviations used:
CTCAE = Common Terminology Criteria for Adverse Events; GIS-pNF = Global Impression of Severity for Plexiform Neurofibroma-related Pain; ICR = independent centralised review; CI = confidence interval; LS = least squares; MV = mean value; N = number of patients evaluated; n = number of patients with (at least one) event; PedsQL = Paediatric Quality of Life Inventory; PN = plexiform neurofibroma; PROMIS = Patient-Reported Outcomes Measurement Information System; PT = preferred term; REiNS = Response Evaluation in Neurofibromatosis and Schwannomatosis; SD = standard deviation; SE = standard error; SOC = system organ class; SAE = serious adverse event; AE = adverse event

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

Children aged 1 year to less than 3 years with symptomatic, inoperable plexiform neurofibromas (PN) associated with neurofibromatosis type 1 (NF1)

Approx. 55 – 95 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 Koselugo (active ingredient: selumetinib) at the following publicly accessible link (last access: 24 June 2026):

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

Treatment with selumetinib should only be initiated and monitored by specialists in internal medicine, haematology and oncology or specialists in paediatrics and adolescent medicine specialising in neuropaediatrics, paediatric haematology and oncology, all of whom are experienced in the treatment of patients with NF1-related tumours.

This medicinal product was approved under "exceptional circumstances". This means that further evidence of the benefit of the medicinal product is anticipated. The EMA will assess new information on this medicinal product at least annually and update the product information as necessary.

4. Treatment costs

Annual treatment costs:

Children aged 1 year to less than 3 years with symptomatic, inoperable plexiform neurofibromas (PN) associated with neurofibromatosis type 1 (NF1)

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Selumetinib	€ 81,237.57 – € 97,443.08[2]
Best supportive care	Different from patient to patient
Appropriate comparator therapy:	
Best supportive care	Different from patient to patient

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

Costs for additionally required SHI services: not applicable

[2] The range of annual treatment costs is determined by the lower limit for children aged 1 year to less than 2 years and the upper limit for children aged 2 years to less than 3 years.

II. The resolution will enter into force on the day of its publication on the G-BA website on 6 August 2026.

The justification for this resolution will be published on the G-BA website at www.g-ba.de.

Berlin, 6 August 2026

Federal Joint Committee
in accordance with Section 91 SGB V
The Chair

Dr Optendrenk