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
Burosumab (new therapeutic indication: FGF23-related hypophosphatemia in tumour-induced osteomalacia, ≥ 1 year)

of 16 February 2023

At its session on 16 February 2023, 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 No. 4 to the information on the benefit assessment of Burosumab in accordance with the resolution of 21 July 2022:

Burosumab

Resolution of: 16 February 2023
Entry into force on: 16 February 2023
Federal Gazette, BAnz AT DD. MM YYYY Bx

New therapeutic indication (according to the marketing authorisation of 25 July 2022):

Crysvita is indicated for the treatment of FGF23-related hypophosphataemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised in children and adolescents aged 1 to 17 years and in adults.

Therapeutic indication of the resolution (resolution of 16 February 2023):

See new therapeutic indication according to marketing authorisation.

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

Patients aged 1 year and above with FGF23-related hypophosphatemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised

Appropriate comparator therapy:

a phosphate replacement and active vitamin D (calcitriol or alfacalcidol) in combination

Extent and probability of the additional benefit of burosumab compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:

Patients aged 1 year and above with FGF23-related hypophosphatemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised

No suitable data submitted.

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	n.a.	There are no assessable data.
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 ∅: There are no usable data for the benefit assessment. n.a.: not assessable		

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

Patients aged 1 year and above with FGF23-related hypophosphatemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised

approx. 60 - 140 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 Crysvita (active ingredient: burosumab) at the following publicly accessible link (last access: 19 December 2022):

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

Treatment with burosumab should only be initiated and monitored by doctors experienced in the therapy of metabolic bone diseases.

4. Treatment costs

Annual treatment costs:

Patients aged 1 year and above with FGF23-related hypophosphatemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised

I. Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Burosumab	€ 66,909.70 - € 1,001,445.26
Appropriate comparator therapy:	
Phosphate replacement and active vitamin D (calcitriol or alfacalcidol) in combination	
Phosphate Children and adolescents Adults	 € 77.78 - € 388.91 € 388.91 - € 622.25
Active vitamin D	
Calcitriol[1]	€ 73.91 - € 513.99
or	
Alfacalcidol Children aged 4 years and below Children aged 5 years and above and adults	 € 189.98 - € 321.38 € 321.38 - € 964.15
	Total
Phosphate + calcitriol Children and adolescents Adults	 Incalculable € 462.82 - € 1,136.25
Phosphate + alfacalcidol	€ 267.76 - € 943.63

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 1 February 2023)

Costs for additionally required SHI services: not applicable

[1] According to the calcitriol product information, as of August 2020, no dosage recommendation can be given for children and adolescents due to the limited data basis available. For this reason, the costs of calcitriol treatment are only presented for adults.

Other SHI services:

Designation of the therapy	Type of service	Costs/ unit	Number/ cycle	Number/ patient/ year	Costs/ patient/ year
Burosumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	13.0 – 26.1	€ 1,300 - € 2,610

5. Medicinal products with new active ingredients according to Section 35a, paragraph 3, sentence 4 SGB V that can be used in a combination therapy with Burosumab

Medicinal products with new active ingredients pursuant to Section 35a, paragraph 3, sentence 4 SGB V are medicinal products with the following new active ingredients which, on the basis of the marketing authorisation under Medicinal Products Act, can be used in a combination therapy with burosumab for the treatment of FGF23-related hypophosphataemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised in children and adolescents aged 1 to 17 years and in adults:

Patients aged 1 year and above with FGF23-related hypophosphatemia in tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours that cannot be curatively resected or localised

- No active ingredient that can be used in a combination therapy that fulfils the requirements of Section 35a, paragraph 3, sentence 4 SGB V.

II. The resolution will enter into force on the day of its publication on the website of the G-BA on 16 February 2023.

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

Berlin, 16 February 2023

Federal Joint Committee (G-BA)
in accordance with Section 91 SGB V
The Chair

Prof. Hecken