SUMMARY

burosumab

CRYSVITA 10 mg, 20 mg and 30 mg

solution for injection in pre-filled syringe

Inclusion on list: First listing

Range supplement

Adopted by the Transparency Committee on 9 October 2024

Summary of opinion

Favourable opinion for reimbursement in:

- the treatment of X-linked hypophosphataemia, in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults.
- 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.

No clinical added value of the new solution for injection in pre-filled syringe forms compared to the forms already available.

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Transparency Committee's conclusions

Clinical Benefit

Treatment of X-linked hypophosphataemia, in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults

- X-linked hypophosphataemia (XLH) is a rare, disabling hereditary disease, with a significant social impact in childhood and adolescence, which may continue into adulthood.
- This is a curative medicinal product.
- The efficacy/adverse effects ratio is high.
- There are therapeutic alternatives.
- This is a first- or second-line treatment in view of the therapies available, as monotherapy, in the treatment of X-linked hypophosphataemia, in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults.

- **Public health benefit**

CRYSVITA (burosumab) is likely to have an additional impact on public health.

The Committee considers that the clinical benefit of CRYSVITA (burosumab) is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the indications and at the MA dosages.

- **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 65%**

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

- High fibroblast growth factor 23 (FGF23)-related tumour-induced osteomalacia is a rare disease characterised by hypophosphataemia due to a kidney phosphate reabsorption disorder. In TIO, a mesenchymal tumour, generally benign and small in size, secretes massive amounts of FGF23. Combined phosphate and 1,25-dihydroxy-vitamin D (calcitriol) deficiencies induce bone matrix demineralisation and severe osteomalacia, with a significant impact on patients' quality of life.
- The proprietary medicinal product CRYSVITA (burosumab) is a curative medicinal product.
- The efficacy/adverse effect ratio of CRYSVITA (burosumab) is high.
- There are medicinal therapeutic alternatives.
- CRYSVITA (burosumab) is a first-line treatment of patients with tumour-induced osteomalacia associated with phosphaturic mesenchymal tumours with high serum FGF23 levels linked with

a non-operable, or partially operated, or non-localised tumour, with persisting clinic signs of chronic hypophosphataemia.

➔ **Public health benefit**

CRYSVITA (burosumab) is unlikely to have an additional impact on public health.

The Committee considers that the clinical benefit of CRYSVITA (burosumab) is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the indications and at the MA dosages.

➔ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 65%**

Clinical Added Value

These medicinal products are range supplements that do not provide any clinical added value (CAV V) compared to the forms already listed.