SUMMARY

burosumab

CRYSVITA 10 mg, 20 mg and 30 mg,

solution for injection

Reassessment at Transparency Committee's request

Original French opinion adopted by the Transparency Committee on 10 January 2024

The legally binding text is the original French opinion version

Transparency Committee's Conclusions

Clinical Benefit

- X-linked hypophosphataemia (XLH) is an incapacitating rare hereditary disorder with substantial social impacts during childhood, which may continue into adulthood.
- This is a curative medicinal product.
- The efficacy/adverse effects ratio is high with a maximum follow-up period of 88 weeks in a study that included children aged one year and over and adolescents in the bone growth phase, with severe forms, failing to respond to conventional treatment, or complex severe forms of XLH (with radiographic signs of bone involvement).
- There are therapeutic alternatives.
- This is a first-line or second-line treatment in view of the therapies available, as monotherapy for the treatment of X-linked hypophosphataemia with radiographic signs of bone involvement in children aged one year and over and adolescents in the bone growth phase.

Public health benefit

In view of:

- the seriousness of the condition and its rare nature,
- the partially met medical need for the treatment of X-linked hypophosphataemia with radiographic signs of bone involvement in children aged one year and over and adolescents in the bone growth phase,
- the partial response provided to the identified need on account of:

- the evidence of an additional impact on morbidity with 88 weeks of follow-up in a phase 3 study and the lack of evidence on an impact on mortality and quality of life,
- an expected impact on delivery of care, and the patient's care and life pathway, considering its different mode of administration from those of the conventional treatment and its safety profile.

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

Considering all of these elements, the Committee deems that the clinical benefit of CRYSVITA (burosumab) 10 mg, 20 mg, 30 mg, solution for injection, is substantial for the treatment of X-linked hypophosphataemia with radiographic signs of bone involvement in children aged one year and over and adolescents in the bone growth phase.

The Committee approves the inclusion of CRYSVITA (burosumab) 10 mg, 20 mg, 30 mg, solution for injection, in the list of covered drugs for outpatients and in the list of covered drugs for inpatients for the treatment of X-linked hypophosphataemia with radiographic signs of bone involvement in children aged one year and over and adolescents in the bone growth phase.

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

Clinical Added Value

In view of:

- the evidence of the superiority of burosumab monotherapy in relation to conventional treatments, a clinically relevant comparator, after 40 weeks of treatment on improvement of a radiology outcome measure,
- the additional effect size deemed to be clinically relevant in a population of paediatric and adolescent patients with severe forms, failing to respond to the conventional treatment,
- the additional follow-up provided by the findings at 64 and 88 weeks which suggest continued efficacy with an acceptable safety profile,
- the lack of usable quality-of-life data in this high-impact condition on this outcome measure,
- the safety profile of burosumab over a longer follow-up period, deemed to be acceptable, compared to the safety profile of conventional treatments, particularly marked by the onset of complications such as ectopic mineralisation, manifested as nephrocalcinosis,
- the administration constraints in respect of the conventional treatment which are particularly impactful for paediatric patients and their close family,

the Committee deems that CRYSVITA 10 mg, 20 mg, 30 mg, solution for injection, provides moderate clinical added value (CAV III) in relation to the conventional treatment for the treatment of X-linked hypophosphataemia with radiographic signs of bone involvement in children aged one year and over and adolescents in the bone growth phase.