Generic Name:
Burosumab
Trade Name:
Crysvita
Company:
Ultragenyx Pharmaceutical
Notes:

FDA has approved burosumab, the first drug approved to treat adults and children aged 1 year and older with x-linked hypophosphatemia (XLH), a rare, inherited form of rickets. XLH causes low levels of phosphorus in the blood. It leads to impaired bone growth and development in children and adolescents and problems with bone mineralization throughout a patient?s life.

Most children with XLH experience bowed or bent legs, short stature, bone pain, and severe dental pain. Some adults with XLH experience persistent discomfort or complications, such as joint pain, impaired mobility, tooth abscesses, and hearing loss.

Safety and efficacy of burosumab were studied in four clinical trials. In the placebo-controlled trial, 94% of adults receiving burosumab once a month achieved normal phosphorus levels, compared with 8% of those receiving placebo. In children, 94% to 100% of patients treated with burosumab every 2 weeks achieved normal phosphorus levels. In both children and adults, X-ray findings associated with XLH improved with burosumab therapy. Comparison of the results to a natural history cohort also provided support for burosumab's effectiveness.

The most common adverse reactions in adults taking burosumab were back pain, headache, restless leg syndrome, decreased vitamin D, dizziness, and constipation. The most common adverse reactions in children were headache, injection site reaction, vomiting, decreased vitamin D, and fever.

Burosumab was granted breakthrough therapy and orphan drug designations, which provides incentives to assist and encourage the development of drugs for rare diseases.

Medication Monitor Categories:
New Drug Approvals