FDA News Release

FDA approves first therapy for rare inherited form of rickets, x-linked hypophosphatemia

For Immediate Release

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Release

The U.S. Food and Drug Administration today approved Crysvita (burosumab), the first drug approved to treat adults and children ages 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.

"XLH differs from other forms of rickets in that vitamin D therapy is not effective," stated Julie Beitz, M.D., director of the Office of Drug Evaluation III in the FDA's Center for Drug Evaluation and Research. "This is the first FDA-approved medication for the treatment of XLH and a real breakthrough for those living with this serious disease."

XLH is a serious disease affecting approximately 3,000 children and 12,000 adults in the United States. 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.

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

The most common adverse reactions in adults taking Crysvita 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 pyrexia (fever).

Crysvita was granted **Breakthrough Therapy (/ForPatients/Approvals/Fast/ucm405397.htm)** designation, under which the FDA provides intensive guidance to the company on efficient drug development, and expedites its review of drugs that are intended to treat serious conditions where clinical evidence shows the drug may represent a substantial improvement over other available therapies. Crysvita also received **Orphan Drug**

(http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm) designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The sponsor is receiving a **Rare Pediatric Disease Priority Review Voucher (https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm)** under a program intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A voucher can be redeemed at a later date to receive **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** of a subsequent marketing application for a different product. This is the 14th Rare Pediatric Disease Priority Review Voucher issued by the FDA since the program began.

The FDA granted approval of Crysvita to Ultragenyx Pharmaceutical Inc.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency is also responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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Inquiries

Media

Theresa Eisenman (mailto:theresa.eisenman@fda.hhs.gov)
301-796-2969

Consumers

888-INFO-FDA

Related Information

- **NIH: X-linked hypophosphatemia (https://rarediseases.info.nih.gov/diseases/12943/x-linked-hypophosphatemia)**
- **FDA: Fast Track, Breakthrough Therapy, Accelerated Approval, and Priority Review (https://www.fda.gov/ForPatients/Approvals/Fast/default.htm)**

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