FDA approves first drug for rare form of rickets

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(HealthDay)—Ultragenyx Pharmaceutical Inc.'s Crysvita (burosumab-

twza) has been approved by the U.S. Food and Drug Administration to treat adults and children ages 1 year and older with x-linked hypophosphatemia (XLH).

A clinical trial found that 94 percent of adults who took Crysvita once a month achieved normal phosphorus levels, compared with 8 percent of those who took a placebo, according to the FDA. In children, 94 to 100 percent of those treated with Crysvita every two weeks achieved normal phosphorus levels.

The most common negative side effects of Crysvita in adults were back pain, headache, restless leg syndrome, decreased vitamin D, dizziness, and constipation. The most common negative side effects in children were headache, injection site reaction, vomiting, decreased vitamin D, and fever, the FDA said.

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

More information: More Information