

Genetic rickets improves more with burosumab than standard care, study finds

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Burosumab, a new injectable medicine to treat X-linked hypophosphatemia (XLH), an inherited form of rickets, demonstrates superior improvements in rickets and other outcomes compared with conventional therapy in an international, phase 3 clinical trial in children. Results from what investigators called the first head-to-head study comparing the new drug with conventional treatment for this rare disease will be presented Sunday at ENDO 2019, the Endocrine Society's annual meeting in New Orleans, La.

"These improvements with burosumab have the potential to change the lives of children with XLH as they grow," said study principal investigator Erik Imel, M.D., associate professor of medicine and pediatrics at Indiana University School of Medicine in Indianapolis, Ind.

XLH affects about 3,000 children and 12,000 adults in the United States, the Food and Drug Administration (FDA) estimates. Typically, XLH causes rickets, bowed legs, bone pain and [short stature](#).

People with XLH have high levels of the hormone fibroblast growth factor 23, or FGF23, which causes low blood levels of phosphorus (hypophosphatemia). Conventional [therapy](#) has long been multiple daily doses of oral phosphate and active vitamin D (e.g. calcitriol), according to Imel.

The study included 61 XLH-affected children ages 1 to 12 years who previously received conventional therapy with oral phosphate and active vitamin D but still had evidence of rickets on X-rays. Patients were randomly assigned to continue this conventional therapy or switch to receive burosumab injections given every two weeks. Radiologists who were unaware of participants' drug assignments reviewed their X-rays and assigned a score for improvement of rickets.

By 40 weeks of treatment, that [improvement](#) was more than two times greater for the burosumab group than the conventional therapy group, Imel noted. Substantial healing of rickets occurred in 72 percent of participants receiving burosumab (21 of 29), the researchers reported, versus only 6 percent in the conventional therapy group (two of 32). Burosumab also reportedly led to greater improvements in leg deformities, height and distance walked in a 6-minute test, as well as larger increases in serum phosphorus and active vitamin D levels.

"We now know the magnitude of benefit from the new medication, burosumab, versus the prior approach with [conventional therapy](#)," Imel said. "This information is critical for doctors making treatment decisions for their patients with XLH."

Provided by The Endocrine Society

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