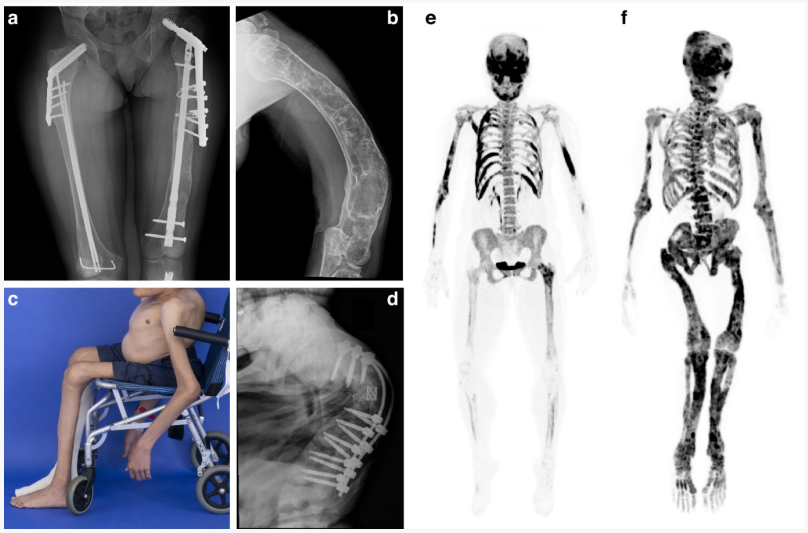
Targeted Therapy Improves Mobility in Children with Rare Bone Disorder

Researchers found that burosumab safely corrected phosphate loss and improved mobility in patients with severe fibrous dysplasia



Clinical images and PET/CT scans from study participants show the wide spectrum of fibrous dysplasia severity, including fractures, bowing deformities, scoliosis, muscle wasting, and extensive skeletal involvement associated with phosphate-wasting disease.

CHINA, May 20, 2026 /EINPresswire.com/ -- A phase 2 clinical trial showed burosumab, a targeted antibody therapy, safely restored phosphate balance in children and adults with fibrous dysplasia, a rare bone disorder associated with fractures and deformities. The therapy reduced abnormal bone turnover markers and improved mobility in severely affected children, including independent walking after full-time wheelchair use. The findings suggest that inhibition of fibroblast growth factor-23 may help reduce disability and improve physical function in patients with phosphate-wasting skeletal disease.

Fibrous dysplasia is a rare skeletal disorder in which normal bone is gradually replaced by weak fibro-osseous tissue, leading to fractures, pain, deformities, and severe mobility impairment. In many patients, diseased bone tissue produces excessive amounts of fibroblast growth factor-23 (FGF23), a hormone that causes phosphate loss through the kidneys. Because phosphate is essential for proper bone mineralization, persistent hypophosphatemia can further weaken already fragile bones and worsen long-term disability. Existing treatment with oral phosphate and vitamin D supplements is often difficult to tolerate and frequently fails to restore normal phosphate levels.

Addressing this challenge, a research team was led by Dr. Alison M. Boyce, who is a Pediatric Endocrinologist at the Metabolic Bone Disorders Unit, National Institute of Dental and Craniofacial Research, National Institutes of Health, USA. The investigators evaluated whether

burosumab, a monoclonal antibody that blocks FGF23 activity, could safely improve phosphate balance and markers of skeletal disease and physical function in patients with fibrous dysplasia.

The phase 2 open-label clinical trial included 12 participants, including seven children and five adults with severe disease burden and hypophosphatemia. Participants received burosumab treatment for 48 weeks, with regular monitoring of phosphate metabolism, bone turnover markers, imaging findings, physical function, and patient-reported outcomes. Their findings were published in Volume 14 of the journal Bone Research on April 27, 2026.

At the start of the study, participants showed extensive skeletal involvement and major physical disability. Many relied on wheelchairs, walkers, or crutches for mobility. Treatment with burosumab successfully restored phosphate levels into the mid-to-upper normal target range in all participants by week 48. The therapy also improved phosphate retention in the kidneys and increased levels of active vitamin D, both essential for healthy bone metabolism. Importantly, alkaline phosphatase, a marker associated with abnormal bone turnover and disease activity, declined substantially during treatment.

The researchers also observed encouraging functional improvements, particularly in pediatric participants. Several children reported reduced pain, less fatigue, and improved physical mobility. Two severely affected children experienced especially dramatic gains. One participant progressed from full-time wheelchair use to independent walking without assistive devices, while another child who had never walked independently was able to walk short distances using a walker after treatment.

“Our findings demonstrate that targeting phosphate levels in the mid to upper normal range can be both safe and clinically meaningful for patients with fibrous dysplasia,” said Dr. Boyce. “The mobility improvements observed in some children suggest that earlier correction of phosphate imbalance may help reduce long-term disability.”

Safety analyses showed that burosumab was generally well tolerated. Most adverse effects were mild and manageable, including temporary episodes of elevated phosphate levels and minor injection-site reactions. Importantly, detailed PET/CT imaging and lesion biopsies showed no evidence that the treatment accelerated lesion growth or increased abnormal tissue activity, addressing a major concern surrounding therapies targeting FGF23 signaling.

The findings may have important implications beyond fibrous dysplasia. The study supports the idea that maintaining optimal phosphate balance could improve outcomes in other disorders driven by excessive FGF23 activity. The results may also encourage collaborations among endocrinologists, bone disease specialists, rehabilitation experts, and rare disease researchers to develop more targeted approaches for skeletal disorders associated with mineral imbalance.

“Children with severe fibrous dysplasia often experience progressive mobility loss during critical

developmental years," Dr. Boyce explained. "Seeing some patients regain meaningful movement and independence provides strong motivation to continue advancing therapies that directly address the biological drivers of disease."

In the short term, burosumab may offer patients and families a more effective alternative to traditional phosphate supplementation, potentially improving quality of life and reducing treatment burden. Over the longer term, the research could help establish targeted biologic therapies as a standard approach for managing rare skeletal diseases associated with phosphate wasting, reducing fractures, deformities, and lifelong disabilities.

Overall, the study demonstrates that burosumab safely restores phosphate homeostasis and may substantially improve physical function in patients with fibrous dysplasia. The findings provide important clinical evidence supporting targeted FGF23 inhibition as a promising strategy for reducing skeletal complications and improving long-term outcomes in this rare and debilitating disorder.

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Reference
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About Dr. Alison M. Boyce from the National Institutes of Health, USA
Dr. Alison M. Boyce is a Pediatric Endocrinologist and Chief of the Metabolic Bone Disorders Unit at the National Institute of Dental and Craniofacial Research, National Institutes of Health, USA. Her research focuses on bone and mineral metabolism, particularly pediatric skeletal disorders. She leads studies investigating bone disease, fibroblast growth factor-23 disorders, connective tissue abnormalities, and skeletal physiology. Dr. Boyce is a faculty member in the NIH Endocrinology fellowship training program, a member of the steering committee for

International Society for Children’s Bone Health, serves as chief Medical Advisor to FD/MAS Alliance, and has authored 120 publications with 4,718 citations.

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