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Therapy for rare bone disorder shows promise in US-based clinical trial

17 April 2023 | News

Medication is the first to reduce bone-weakening process in patients with fibrous dysplasia



A clinical trial at the National Institutes of Health (NIH), in the US, found that a medication, denosumab, significantly reduced abnormal bone turnover in adults with fibrous dysplasia, a rare disease marked by weak and misshapen bones.

Bone turnover, a process in which old bone is continuously replaced with new bone, is unusually accelerated in fibrous dysplasia and contributes to bone abnormalities.

The study of eight participants was carried out by researchers from the National Institute of Dental and Craniofacial Research (NIDCR) and the NIH Clinical Center. The results showed that denosumab may improve patients' quality of life by enabling healthy bone formation.

Fibrous dysplasia stems from gene mutations that cause scar-like (fibrous) tissue to replace healthy bone starting in early childhood. These fibrous lesions, which are marked by accelerated bone turnover, weaken bones, leading to bone deformities, fractures, physical disabilities, and pain. In some cases, the lesions can press up against organs and nerves, impairing functions like vision and breathing.

Denosumab is a medication licensed by the US Food and Drug Administration to treat bone problems from osteoporosis and cancer.

This trial is the culmination of 25 years of clinical research at NIDCR to understand the underlying mechanisms of fibrous dysplasia and to identify promising treatments.