Cyclic Administration of Pamidronate in Children with Severe Osteogenesis Imperfecta

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ABSTRACT

Background Severe osteogenesis imperfecta is a disorder characterized by osteopenia, frequent fractures, progressive deformity, loss of mobility, and chronic bone pain. There is no effective therapy for the disorder. We assessed the effects of treatment with a bisphosphonate on bone resorption.

Methods In an uncontrolled observational study involving 30 children who were 3 to 16 years old and had severe osteogenesis imperfecta, we administered pamidronate intravenously (mean [±SD] dose, 6.8±1.1 mg per kilogram of body weight per year) at 4-to-6-month intervals for 1.3 to 5.0 years. Clinical status, biochemical characteristics reflecting bone turnover, the bone mineral density of the lumbar spine, and radiologic changes were assessed regularly during treatment.

Results Administration of pamidronate resulted in sustained reductions in serum alkaline phosphatase concentrations and in the urinary excretion of calcium and type I collagen *N*-telopeptide. There was a mean annualized increase of 41.9±29.0 percent in bone mineral density, and the deviation of bone mineral density from normal, as indicated by the z score, improved from –5.3±1.2 to –3.4±1.5. The cortical width of the metacarpals increased by 27.0±20.2 percent per year. The increases in the size of the vertebral bodies suggested that new bone had formed. The mean incidence of radiologically confirmed fractures decreased by 1.7 per year (P<0.001). Treatment with pamidronate did not alter the rate of fracture healing, the growth rate, or the appearance of the growth plates. Mobility and ambulation improved in 16 children and remained unchanged in the other 14. All the children reported substantial relief of chronic pain and fatigue.

Conclusions In children with severe osteogenesis imperfecta, cyclic administration of intravenous pamidronate improved clinical outcomes, reduced bone resorption, and increased bone density.

Source Information

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