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İmigluserazla ERT Gaucher'de kemik mineral yoğunluğunu artırır.

Wenstrup RJ et al. Effect of enzyme replacement therapy with imiglucerase on BMD in type 1 Gaucher disease. *J Bone Miner Res* 2007;22:119-26.

The effect of ERT with imiglucerase on BMD in type 1 GD was studied using BMD data from the International Collaborative Gaucher Group Gaucher Registry. Data were analyzed for 160 untreated patients and 342 ERT-treated patients. Imiglucerase significantly improves BMD in patients with GD, with 8 years of ERT leading to normal BMD.

Introduction: The objective was to determine the effect of enzyme replacement therapy (ERT; Cerezyme, imiglucerase) on BMD in type 1 Gaucher disease (GD).

Materials and Methods: The study population included all adults (men, 18-70 years; women, 18-50 years) enrolled in the International Collaborative Gaucher Group (ICGG) Gaucher Registry for whom lumbar spine BMD measurements were available. BMD data with up to 8 years of follow-up were analyzed for 160 patients who received no ERT and 342 patients treated with ERT alone. BMD was assessed by DXA of the lumbar spine. Z scores for patients with GD were compared with a reference population. From the model's estimate, percent of patients by age and sex with osteoporosis (T score ≤ -2.5) were calculated.

Results: DXA Z scores for patients with GD in the no ERT (untreated) group were significantly below normal (y intercept = -0.820 Z score units, $p < 0.001$) and remained -1 SD below the reference population over time (slope = -0.010 Z score units per year, $p = 0.68$). The DXA Z scores for patients with GD who received ERT at a dose of 60 U/kg/2 weeks were significantly lower than the reference population at baseline (y-intercept = -1.177 Z score units, $p < 0.001$), but improved significantly over time (slope = $+0.132$ Z score units per year, $p < 0.001$). A significant dose-response relationship was noted for the ERT group, with the slopes for the three main dosing groups of 15 , 30 , and 60 U/kg/2 weeks of $+0.064$, $+0.086$, and $+0.132$ Z score units per year, respectively. The BMD of patients with GD treated with ERT increased -0.12 (60 U/kg/2 weeks), -0.48 (30 U/kg/2 weeks), and -0.66 (15 U/kg/2 weeks) SD of the mean of the reference population after 8 years of ERT, respectively. The BMD of patients with GD treated with ERT increased more than untreated, approaching the reference population. Estimated risk of osteoporosis of this GD population, if left untreated, ranged from -10 to 30% in women and 10% to 25% in men.

Conclusions: ERT with imiglucerase (Cerezyme) may increase BMD in patients with GD. Response to treatment with imiglucerase is slower for BMD than for hematologic and visceral aspects of GD. A normal (age- and sex-adjusted) BMD should be a therapeutic goal for patients with type 1 GD.

