Cerezyme® 30 YIL

# 30 YILDAN 30 MAKALE

## 2008
## İki yıllık sonuçlara göre imigluseraz kemik bulgularını iyileştirir.

***Sims KB et al. Improvement of bone disease by imiglucerase (Cerezyme) therapy in patients with skeletal manifestations of type 1 Gaucher disease: results of a 48-month longitudinal cohort study. Clin Genet 2008;73:430-40.***

Progressive skeletal disease accounts for some of the most debilitating complications of type 1 Gaucher disease. In this 48-month, prospective, non-randomized, open-label study of the effect of enzyme replacement therapy on bone response, 33 imiglucerase-naïve patients (median age 43 years with one or more skeletal manifestations such as osteopenia, history of bone crisis, or other documented bone pathology) received imiglucerase 60 U/kg/2 weeks. Substantial improvements were observed in bone pain (BP), bone crises (BC), and bone mineral density (BMD). Improvements in BP were observed at 3 months ($p < 0.001$ vs baseline) and continued progressively throughout the study, with 39% of patients reporting pain at 48 months vs 73% at baseline. Eleven of the 13 patients with a pre-treatment history of BC had no recurrences. Biochemical markers for bone formation increased; markers for bone resorption decreased. Steady improvement of spine and femoral neck BMD, measured using dual-energy X-ray absorptiometry was noted. Mean Z score for spine increased from −0.72 ± 1.302 at baseline to near-normal levels (−0.09 ± 1.503) by month 48 ($p = 0.042$) and for femoral neck from −0.59 ± 1.352 to −0.17 ± 1.206 ($p = 0.035$) at month 36. This increase was sustained at 48 months. With imiglucerase treatment, patients should anticipate resolution of BC, rapid improvement in BP, increases in BMD, and decreased skeletal complications.

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