

30 YILDAN 30 MAKALE

2007 ERT'nin kemikte etkili olduđu gösterilmiştir.

Charrow J et al. The effect of enzyme replacement therapy on bone crisis and bone pain in patients with type 1 Gaucher disease. Clin Genet 2007;71:205-11.

The effect of enzyme replacement therapy (ERT) on bone crisis and bone pain was investigated in patients with Gaucher disease (GD) type 1 followed over 4 years. Data from the International Collaborative Gaucher Group Gaucher Registry were used. Only patients with bone crisis and/or bone pain data for 1 year prior to ERT, and for each of 3 years after the start of ERT, were included. Bone crises were reported in 17% of patients during the year before starting ERT. The frequencies of bone crises decreased to 5%, <1% and 3% for 1, 2, and 3 years after initiation of treatment, respectively ($p < 0.0001$). Bone pain followed a similar pattern of response. Bone pain was reported in 49% of patients the year before treatment and decreased to 30% in the first year, 29% in the second year, and 30% in the third year of ERT ($p < 0.0001$). ERT is associated with a reduction in bone crisis and bone pain in patients with GD type 1. This study shows that significant improvements in symptoms of skeletal disease are achievable clinical outcomes and treatment goals in GD type 1.

