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**İlk uzun dönemli sonuçlar
ERT'nin uzun dönemli güvenlilik
ve etkililiğini belgelemiştir.**

Niederau C, vom Dahl S, Häussinger D. First long-term results of imiglucerase therapy of type 1 Gaucher disease. Eur J Med Res 1998;3:25-30.

Background/Aims: In the early 1990s, enzyme replacement therapy with modified placental glucocerebrosidase (alglucerase, Genzyme Corporation, Cambridge, MA, USA) was shown to arrest or reverse complications and to improve quality of life in patients with type 1 Gaucher's disease. More recently, modified recombinant glucocerebrosidase (imiglucerase, Genzyme Corporation) has been shown to be safe, effective and clinically equivalent to alglucerase by two studies which presented data for 12 months' follow-up. This case report, with 30 months' follow-up, represents the first publication of long-term results of imiglucerase therapy of type 1 Gaucher's disease in Europe.

Methods: Retrospective analysis of safety and efficacy of 30 months' imiglucerase infusions, 40 U/kg body weight every 2 weeks for 17 months, then 60 U/kg every 2 weeks for 13 months, in an elderly male patient with severe type 1 Gaucher's disease.

Results: No adverse reactions occurred, and anti-imiglucerase antibody assay was negative at 17 months. Clinically, the patient responded rapidly and markedly. Within several months, bone pain decreased notably, enabling him to abandon crutches. Abdominal pain abated, fatigue decreased and physical fitness and general wellbeing improved. Nosebleeds and hematomas disappeared. Investigations revealed reduced hepatomegaly and splenomegaly.

Conclusions: This case documents long-term safety and efficacy of recombinant enzyme replacement in type 1 Gaucher's disease.

