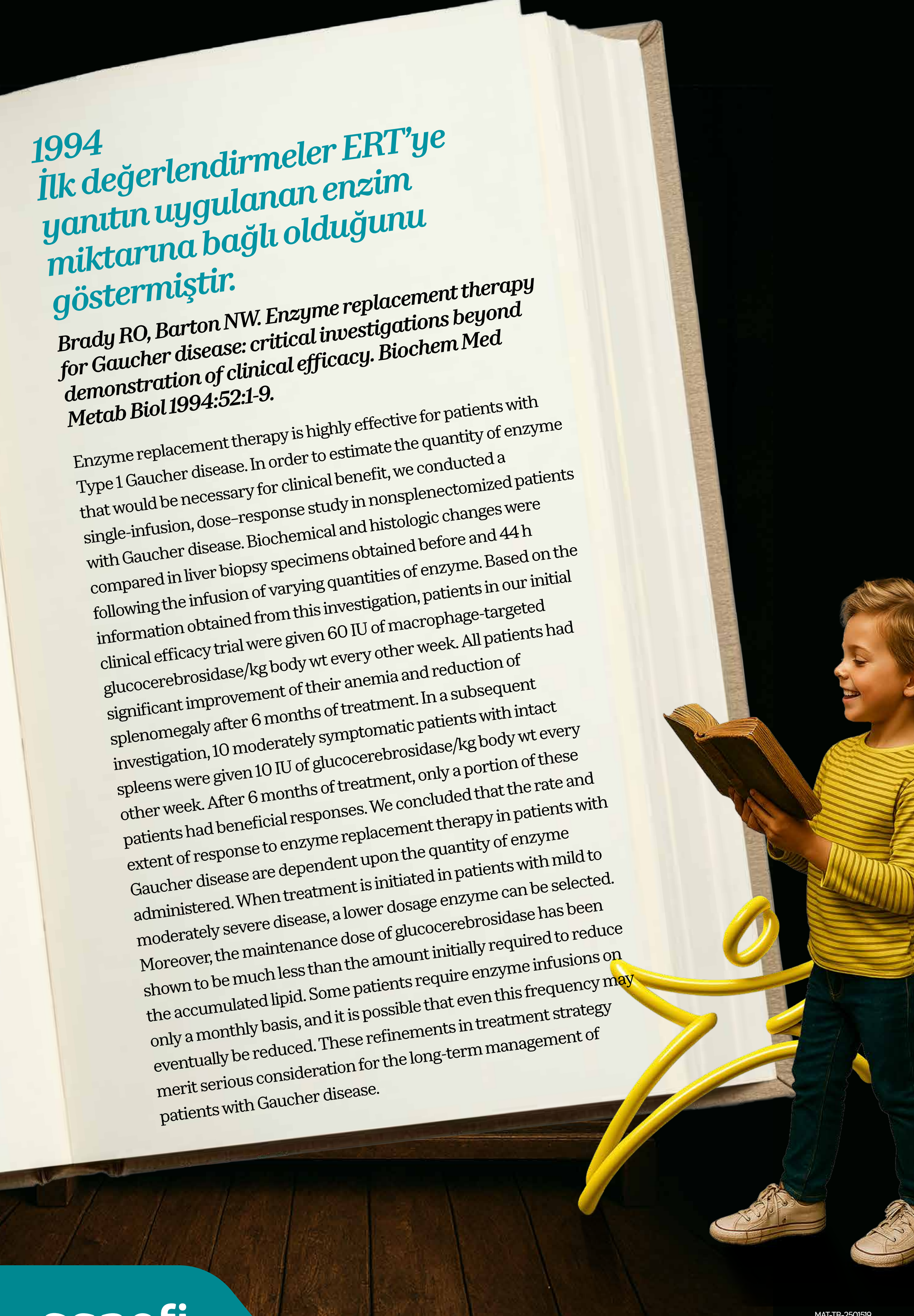
Cerezyme® 30 YIL

30 YILDAN 30 MAKALE

1994
İlk değerlendirmeler ERT'ye yanıtın uygulanan enzim miktarına bağlı olduğunu göstermiştir.

Brady RO, Barton NW. Enzyme replacement therapy for Gaucher disease: critical investigations beyond demonstration of clinical efficacy. Biochem Med Metab Biol 1994:52:1-9.

Enzyme replacement therapy is highly effective for patients with Type 1 Gaucher disease. In order to estimate the quantity of enzyme that would be necessary for clinical benefit, we conducted a single-infusion, dose–response study in nonsplenectomized patients with Gaucher disease. Biochemical and histologic changes were compared in liver biopsy specimens obtained before and 44 h following the infusion of varying quantities of enzyme. Based on the information obtained from this investigation, patients in our initial clinical efficacy trial were given 60 IU of macrophage-targeted glucocerebrosidase/kg body wt every other week. All patients had significant improvement of their anemia and reduction of splenomegaly after 6 months of treatment. In a subsequent investigation, 10 moderately symptomatic patients with intact spleens were given 10 IU of glucocerebrosidase/kg body wt every other week. After 6 months of treatment, only a portion of these patients had beneficial responses. We concluded that the rate and extent of response to enzyme replacement therapy in patients with Gaucher disease are dependent upon the quantity of enzyme administered. When treatment is initiated in patients with mild to moderately severe disease, a lower dosage enzyme can be selected. Moreover, the maintenance dose of glucocerebrosidase has been shown to be much less than the amount initially required to reduce the accumulated lipid. Some patients require enzyme infusions on only a monthly basis, and it is possible that even this frequency may eventually be reduced. These refinements in treatment strategy merit serious consideration for the long-term management of patients with Gaucher disease.



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