Cerezyme® 30 YIL

30 YILDAN 30 MAKALE

2008
8 yıllık sonuçlara göre pediyatrik hastalarda ERT ile klinik parametrelerden çoğu normal ya da normale yakındır.

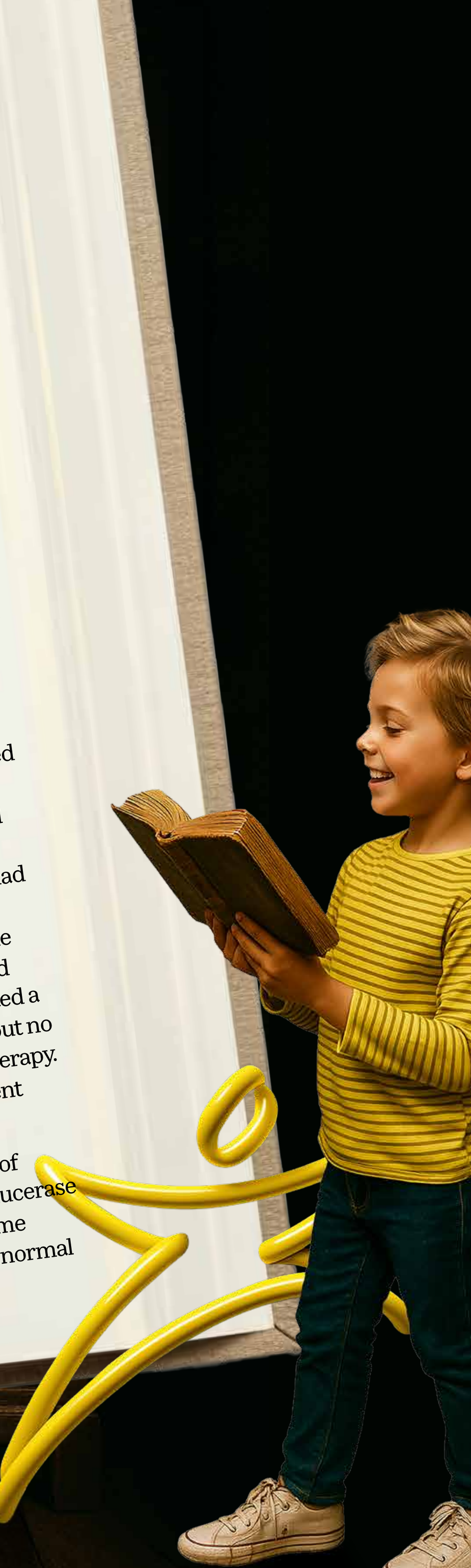
Andersson H et al. Eight-year clinical outcomes of long-term enzyme replacement therapy for 884 children with Gaucher disease type. Pediatrics 2008;122:1182-90.

OBJECTIVE. The goal was to analyze the clinical responses to enzyme replacement therapy with alglucerase or imiglucerase in a large international cohort of children with Gaucher disease type 1.

METHODS. Anonymized data from 884 children in the International Collaborative Gaucher Group Gaucher Registry were analyzed to determine the effects of long-term enzyme replacement therapy with alglucerase or imiglucerase on hematologic and visceral manifestations, linear growth, and skeletal disease. The parameters measured were hemoglobin levels, platelet counts, spleen and liver volumes, z scores for height and bone mineral density, and reports of bone pain and bone crises.

RESULTS. The median height z score for the study population was –1.4 at baseline. After 8 years of treatment, the median height approximated the median value for the normal population. Anemia, although not severe, was present in >50% of patients at baseline and resolved for all patients after 8 years of treatment. More than 50% of patients had platelet counts of <100 000 platelets per mm^3 at baseline, but >95% had platelet counts above this level after 8 years of treatment. Liver and spleen volumes decreased over 8 years of treatment. The mean bone mineral density z score was –0.34 at baseline, and values normalized within 6.6 years of treatment. Seventeen percent of patients reported a bone crisis before treatment and in the first 2 years of treatment, but no bone crises were reported after 2 years of enzyme replacement therapy. Few patients (2.5%) without bone crises before enzyme replacement therapy had a crisis after start of treatment.

CONCLUSIONS. These longitudinal data quantitate the benefits of continuous enzyme replacement therapy with alglucerase/imiglucerase for children with Gaucher disease type 1. Within 8 years of enzyme replacement therapy, most clinical parameters studied became normal or nearly normal.



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