Cerezyme® 30 YIL

30 YILDAN 30 MAKALE

2007
ERT'ye ara vermekten kaçınılmalıdır.

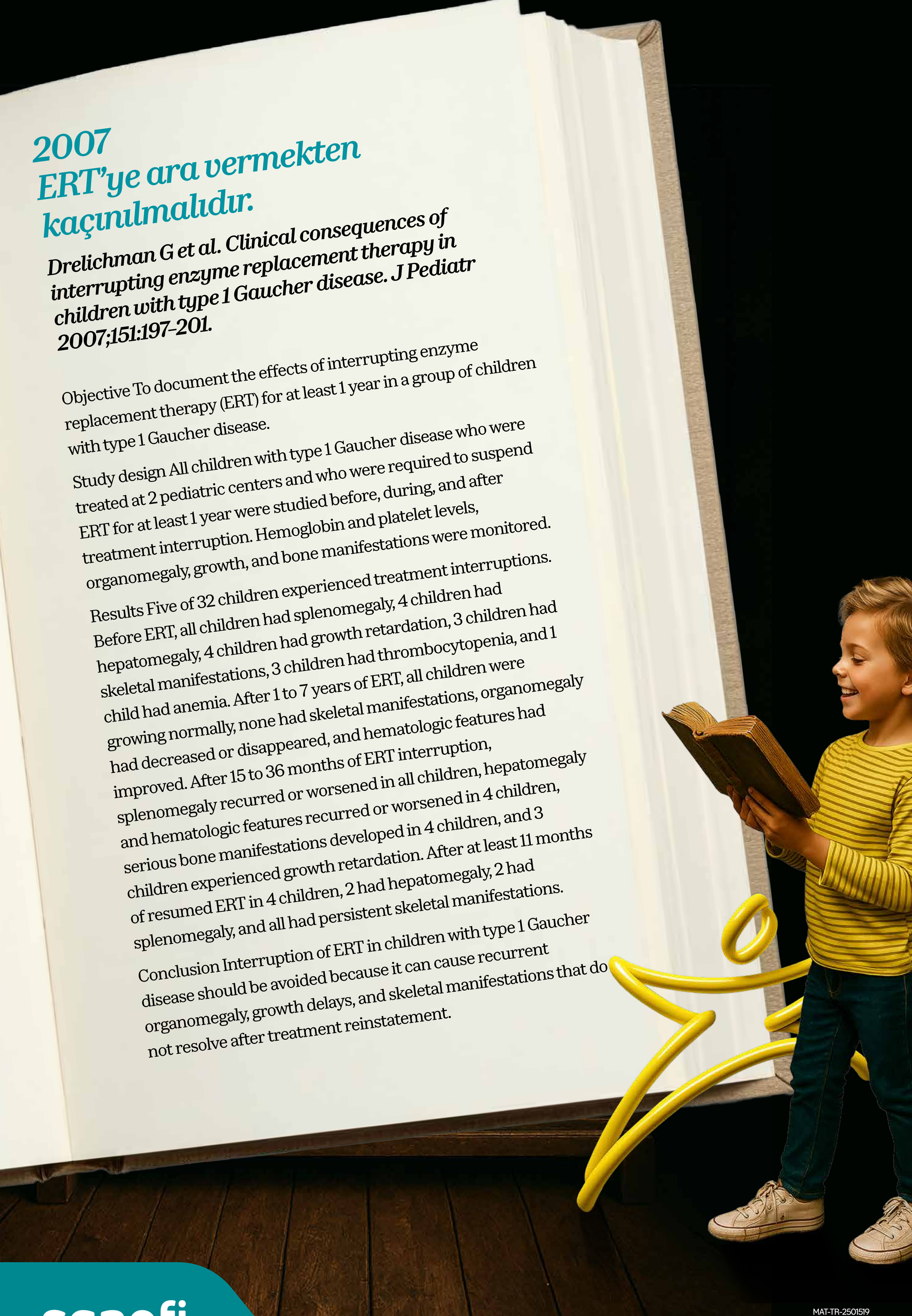
Drelichman G et al. Clinical consequences of interrupting enzyme replacement therapy in children with type 1 Gaucher disease. J Pediatr 2007;151:197–201.

Objective To document the effects of interrupting enzyme replacement therapy (ERT) for at least 1 year in a group of children with type 1 Gaucher disease.

Study design All children with type 1 Gaucher disease who were treated at 2 pediatric centers and who were required to suspend ERT for at least 1 year were studied before, during, and after treatment interruption. Hemoglobin and platelet levels, organomegaly, growth, and bone manifestations were monitored.

Results Five of 32 children experienced treatment interruptions. Before ERT, all children had splenomegaly, 4 children had hepatomegaly, 4 children had growth retardation, 3 children had skeletal manifestations, 3 children had thrombocytopenia, and 1 child had anemia. After 1 to 7 years of ERT, all children were growing normally, none had skeletal manifestations, organomegaly had decreased or disappeared, and hematologic features had improved. After 15 to 36 months of ERT interruption, splenomegaly recurred or worsened in all children, hepatomegaly and hematologic features recurred or worsened in 4 children, serious bone manifestations developed in 4 children, and 3 children experienced growth retardation. After at least 11 months of resumed ERT in 4 children, 2 had hepatomegaly, 2 had splenomegaly, and all had persistent skeletal manifestations.

Conclusion Interruption of ERT in children with type 1 Gaucher disease should be avoided because it can cause recurrent organomegaly, growth delays, and skeletal manifestations that do not resolve after treatment reinstatement.



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MAT-TR-2501519