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ERT Gaucher hastası çocuk ve ergenlerin büyümesini normalleştirir.

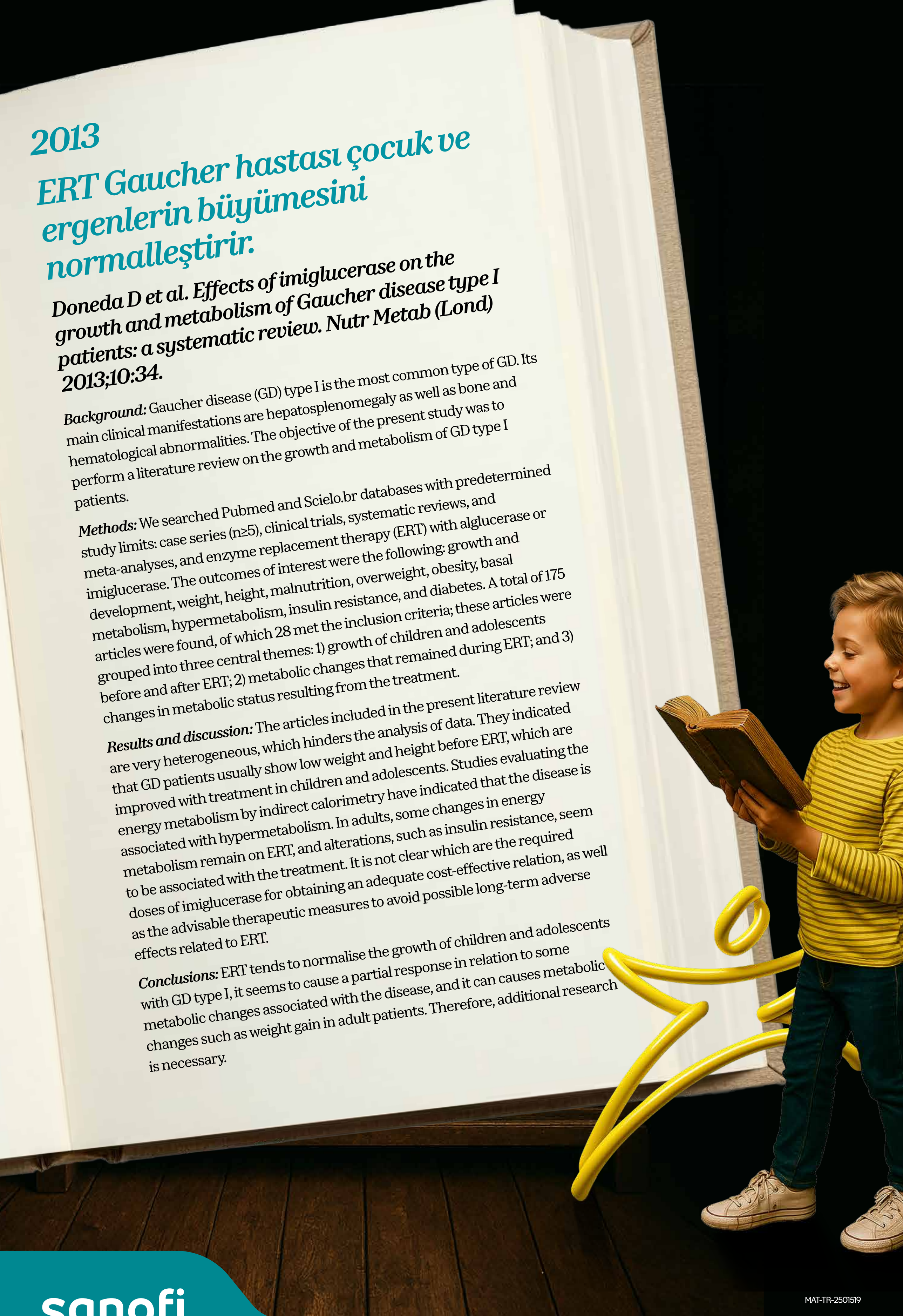
Doneda D et al. Effects of imiglucerase on the growth and metabolism of Gaucher disease type I patients: a systematic review. Nutr Metab (Lond) 2013;10:34.

Background: Gaucher disease (GD) type I is the most common type of GD. Its main clinical manifestations are hepatosplenomegaly as well as bone and hematological abnormalities. The objective of the present study was to perform a literature review on the growth and metabolism of GD type I patients.

Methods: We searched Pubmed and Scielo.br databases with predetermined study limits: case series (n≥5), clinical trials, systematic reviews, and meta-analyses, and enzyme replacement therapy (ERT) with alglucerase or imiglucerase. The outcomes of interest were the following: growth and development, weight, height, malnutrition, overweight, obesity, basal metabolism, hypermetabolism, insulin resistance, and diabetes. A total of 175 articles were found, of which 28 met the inclusion criteria; these articles were grouped into three central themes: 1) growth of children and adolescents before and after ERT; 2) metabolic changes that remained during ERT; and 3) changes in metabolic status resulting from the treatment.

Results and discussion: The articles included in the present literature review are very heterogeneous, which hinders the analysis of data. They indicated that GD patients usually show low weight and height before ERT, which are improved with treatment in children and adolescents. Studies evaluating the energy metabolism by indirect calorimetry have indicated that the disease is associated with hypermetabolism. In adults, some changes in energy metabolism remain on ERT, and alterations, such as insulin resistance, seem to be associated with the treatment. It is not clear which are the required doses of imiglucerase for obtaining an adequate cost-effective relation, as well as the advisable therapeutic measures to avoid possible long-term adverse effects related to ERT.

Conclusions: ERT tends to normalise the growth of children and adolescents with GD type I, it seems to cause a partial response in relation to some metabolic changes associated with the disease, and it can causes metabolic changes such as weight gain in adult patients. Therefore, additional research is necessary.



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