Cerezyme® 30 YIL

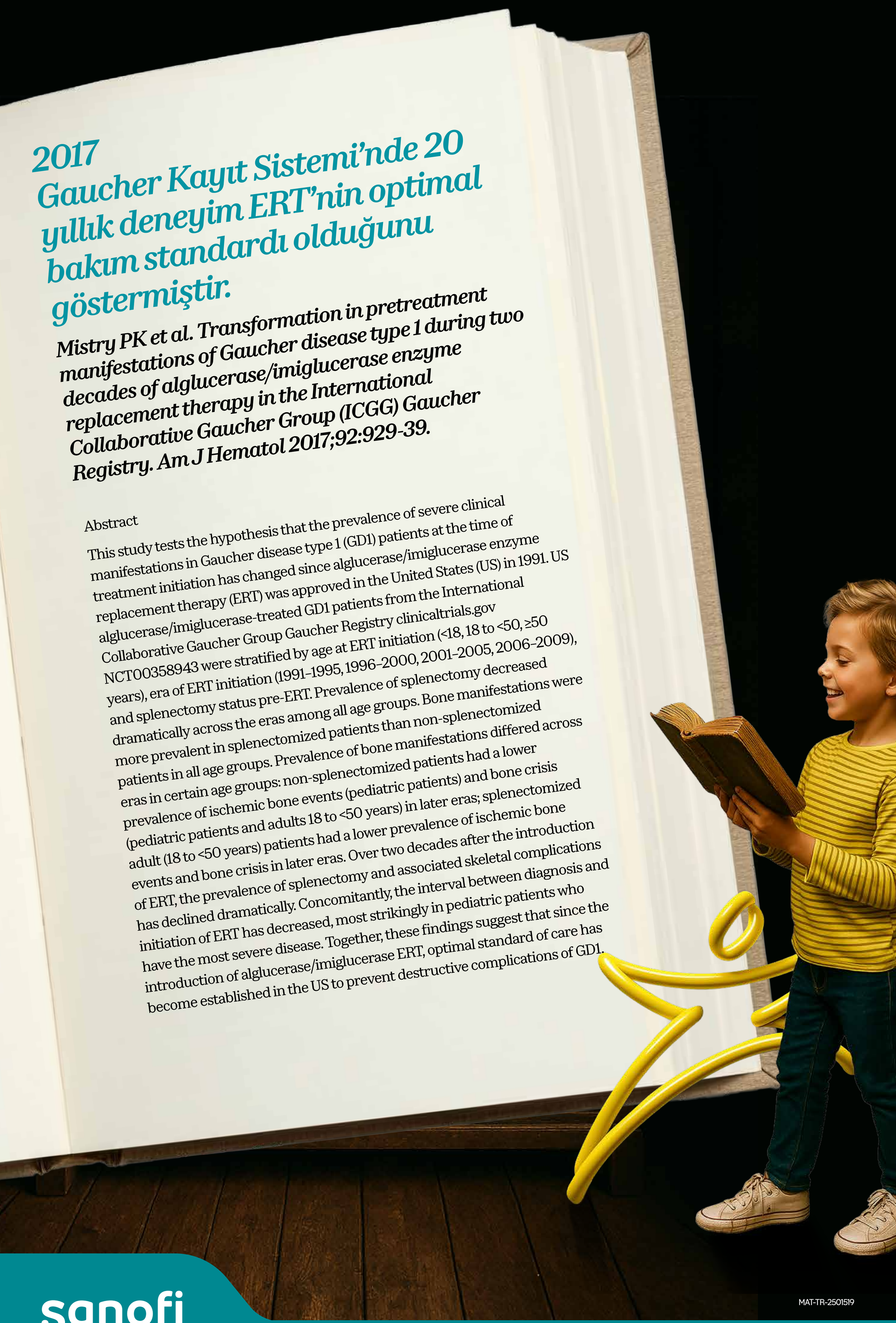
30 YILDAN 30 MAKALE

2017 Gaucher Kayıt Sistemi'nde 20 yıllık deneyim ERT'nin optimal bakım standardı olduğunu göstermiştir.

Mistry PK et al. Transformation in pretreatment manifestations of Gaucher disease type 1 during two decades of alglucerase/imiglucerase enzyme replacement therapy in the International Collaborative Gaucher Group (ICGG) Gaucher Registry. Am J Hematol 2017;92:929-39.

Abstract

This study tests the hypothesis that the prevalence of severe clinical manifestations in Gaucher disease type 1 (GD1) patients at the time of treatment initiation has changed since alglucerase/imiglucerase enzyme replacement therapy (ERT) was approved in the United States (US) in 1991. US alglucerase/imiglucerase-treated GD1 patients from the International Collaborative Gaucher Group Gaucher Registry clinicaltrials.gov NCT00358943 were stratified by age at ERT initiation (<18, 18 to <50, ≥50 years), era of ERT initiation (1991–1995, 1996–2000, 2001–2005, 2006–2009), and splenectomy status pre-ERT. Prevalence of splenectomy decreased dramatically across the eras among all age groups. Bone manifestations were more prevalent in splenectomized patients than non-splenectomized patients in all age groups. Prevalence of bone manifestations differed across eras in certain age groups: non-splenectomized patients had a lower prevalence of ischemic bone events (pediatric patients) and bone crisis (pediatric patients and adults 18 to <50 years) in later eras; splenectomized adult (18 to <50 years) patients had a lower prevalence of ischemic bone events and bone crisis in later eras. Over two decades after the introduction of ERT, the prevalence of splenectomy and associated skeletal complications has declined dramatically. Concomitantly, the interval between diagnosis and initiation of ERT has decreased, most strikingly in pediatric patients who have the most severe disease. Together, these findings suggest that since the introduction of alglucerase/imiglucerase ERT, optimal standard of care has become established in the US to prevent destructive complications of GD1.



sanofi

MAT-TR-2501519