Cerezyme® 30 YIL

30 YILDAN 30 MAKALE

1997
1990'dan itibaren elde edilen kanıtlar Gaucher'de ERT'nin yüksek düzeyde yararlı olduğunu göstermiştir.

Brady RO. Gaucher's disease: past, present and future. Ballieres Clin Haematol 1997;10:621–34.

A patient with what is now known as Gaucher's disease was first described by P. C. E. Gaucher in 1882. Fifty years later, Aghion reported that patients with this condition accumulated a sphingoglycolipid called glucocerebroside. Considerably more time was required for the demonstration by Brady and co-workers in 1964 that Gaucher's disease was due to reduced activity of a β-glucosidase called glucocerebrosidase. This information provided the basis for the development of reliable diagnostic tests, detection of most of the carriers of this disorder and the prenatal diagnosis of this condition. Evidence was presented in 1990 and 1991 indicating the highly beneficial effects of enzyme replacement therapy in patients with Gaucher's disease. Gene therapy for Gaucher's disease was initiated in 1995. While little indication of success was obtained in this inaugural attempt, it is expected that improvements in this technology will provide a permanent cure for patients with this disorder.

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