Cerezyme® 30 YIL

30 YILDAN 30 MAKALE

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20 yıllık deneyim imigluserazın etkili bir uzun dönemli tedavi olduğunu göstermiştir.

Weinreb NJ et al; International Collaborative Gaucher Group (ICGG) Gaucher Registry (NCT00358943) investigators. Gaucher disease type 1 patients from the ICGG Gaucher Registry sustain initial clinical improvements during twenty years of imiglucerase treatment. Mol Genet Metab 2021;132:100-111.

Background: Alglucerase enzyme replacement therapy was approved for Gaucher disease (GD) in the United States in 1991; imiglucerase in 1994. We report hematologic, visceral, bone pain, bone crisis, height, weight, and Body Mass Index (BMI) outcomes in patients treated for 20 (±3) years with subset analyses based on pretreatment severity, genotype, and age at treatment initiation.

Methods: GD type 1 (GD1) patients in the ICGG Gaucher Registry with complete sets of baseline, 10-year, and 20-year data are included (N = 475). Ten-year and 20-year data are compared to pre-treatment baseline, stratified by splenectomy status.

Results: Non-splenectomized patients: Improvements observed at 10 years were maintained at 20 years for most outcomes. Mean changes from baseline at 10 and 20 years, respectively, were: spleen volume: 18.2 multiples of normal (MN) to 5.1 MN and 4.2 MN; liver volume: 1.8 MN to 1.0 MN and 1.0 MN; hemoglobin: 11.4 g/dL to 13.7 g/dL and 13.8 g/dL; platelet count: 91.6 × 10^9/L to 168.0 × 10^9/L and 169.1 × 10^9/L; without bone crisis: 85.0% to 98.2% and 96.5%; without bone pain: 52.5% to 72.0% at 10 years, no significant change at 20 years (58.5%). Splenectomized patients: significant changes were observed in liver volume: 2.3 MN to 1.1 MN and 1.0 MN; hemoglobin: 11.7 g/dL to 13.3 g/dL and 13.4 g/dL; platelet count: 229.1 × 10^9/L to 288.1 × 10^9/L and 257.0 × 10^9/L; without bone crisis: 52.2% to 91.3% and 100%; without bone pain: 16.3% to 30.6% (not significant) and 46.9%. Similar results were found in each of the subset analyses. Patients who start treatment during childhood have normal weight and height in young adulthood. Many treated adult patients are overweight or obese; however, this is consistent with BMI trends observed in the general population. After 1–2 years, the average biweekly imiglucerase dose is ~40 units/kg body weight.

Conclusion: Imiglucerase is an effective, long-term treatment for GD1. In a long-term observational setting, improvements seen during early treatment years are sustained by continuing treatment for 20 years, except for bone pain in non-splenectomized patients. These results are consistent when analyzed by different patient subsets, including by disease severity.

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