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Gaucher'de ERT ile yaşam kalitesi, diğer kronik hastalıklara kıyasla daha fazla artmaktadır.

Damiano AM, Pastores GM, Ware JE Jr. The health related quality of life of adults with Gaucher's disease receiving enzyme replacement therapy: results from a retrospective study. *Qual Life Res* 1998;7:373-86.

Few studies have reported on the effect of Gaucher's disease on patient-reported, health-related quality of life (HRQoL) and we do not know how the HRQoL burden of Gaucher's disease compares to that of other chronic conditions, what areas of HRQoL are most affected or how the course of change in HRQoL compares with that observed for other conditions for the general adult population. The purpose of this study was to estimate (1) the HRQoL burden associated with Gaucher's disease managed by enzyme replacement therapy (ERT), (2) recalled changes in HRQoL since ERT initiation and (3) risk factors predictive of HRQoL outcomes. We sampled 212 patients with Gaucher's disease recruited from 146 physicians prescribing ERT in the US. The patients were at least 14 years of age and had been on ERT from 1 to 51 months. The mean (SD) age of the participant was 45 (17) years. Forty-nine percent had had a prior splenectomy and 26% had had a joint replacement. We administered the SF-36 Health Survey (SF-36) and three questions about changes in physical, mental and general HRQoL since starting ERT. The patients with Gaucher's disease scored significantly worse than the age- and gender-adjusted US norms on five of the eight SF-36 subscales ($p < 0.05$). Age ($p < 0.001$) and joint replacement ($p < 0.001$) were negatively associated with physical health. The presence of an intact spleen ($p < 0.01$) and a longer duration of ERT ($p < 0.01$) were associated with better mental health. When asked about changes in HRQoL since starting ERT, at least half of the patients reported fewer limitations in physical activities (53%), better emotional health perceptions (77%) and less negative emotions (49%) at the time of the interview. Patients who had been receiving ERT for approximately 1 year recalled four and five times more improvement in general HRQoL in comparison with recalled changes over a 4 year period among adults in the general population ($p < 0.01$) and a congestive heart failure population ($p < 0.01$), respectively. Odds ratios (ORs) revealed that female patients were more likely to report improvements in general HRQoL than males (OR = 4.50 and 95% CI = 2.19-9.25) and 45 year old patients were less likely to report improvements than 35 year olds (OR = 0.76 and 95% CI = 0.62-0.94). Relative to patients who had been receiving ERT for 1 year, those who had been receiving ERT for 2 and 4 years were 1.40 (95% CI = 1.06-1.84) and 2.75 (95% CI = 1.20-6.27) times more likely to report improvements in general HRQoL, respectively. In summary, patients with Gaucher's disease on ERT reported an improvement in HRQoL that was greater than that reported by patients with other chronic diseases. However, Gaucher's patients treated for up to 51 months scored below equivalent adults in the general population. The risk factors, including age and history of splenectomy and joint replacement, warrant further study. Standardized HRQoL measures are likely to prove useful in understanding better the outcomes from the Gaucher's patient's perspective.

