



Effect of Enzyme Replacement Therapy on Disease Burden Biomarkers in Gaucher's Disease

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Abstract:

Aims: We evaluate the effect of enzyme replacement therapy "ERT" on the plasma levels of some biochemical markers of the disease burden, in pediatric patients with Gaucher's disease "GD", in the form of plasma chitotriosidase "ChT", total acid phosphatase activity, ferritin and globulin to evaluate the therapeutic monitoring efficacy of such biomarkers. **Methodology:** A cross sectional case control study, carried out on 26 GD pediatric patients, divided into group A (13): On ERT and group B (13): Not receiving ERT, and in addition to 20 healthy age and sex matched controls. ELISA assays of plasma ChT and ferritin, colorimetric assays of plasma total acid phosphatase activity and globulin, were done for all groups, while, plasma proteins electrophoresis was done for GD patients only. **Results:** Significant higher plasma levels of ChT, total acid phosphatase activity, ferritin and globulin among GD not receiving ERT versus both GD on ERT and Control group. Positive correlation between plasma ferritin and total acid phosphatase activity ($r = 0.465$ and P-value

Keywords:

Disease burden markers; Gaucher's disease; ERT.

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