

# **98 The outcome of haemathological parameters and organomegaly under Imyglucerase treatment in Romanian Type 1 Gaucher patients**

## **Centres of expertise for rare diseases**

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**Keywords :**

Type 1 Gaucher disease, Romanian patients, Imyglucerase treatment, outcome

**Summary :**

ERT with Imyglucerase in Romanian type 1 Gaucher patients for a mean of 28 month shows a significant improvement of haematological parameters and organomegaly

**Text :**

**Aim:** This study aimed to evaluate the outcome of haemathological parameters and organomegaly in Romanian type 1 Gaucher patients under Imyglucerase treatment, which has recently been available in our country.

**Patients and methods:** 30 patients with type 1 Gaucher disease (age: 31.74 + 12.02 years; range: 13 – 53) and 1 patient with type 3 have been treated by now, with 20–60 units/Kg/dose of Imyglucerase, every two weeks, for a minimum of 6 month (28.43 + 11.39 month). Haemoglobin (g/dl), platelet count (/mm<sup>3</sup>), spleen and liver volume were assessed every 6 month.

**Results:** Haemoglobin level has a significant increased from the start of ERT (11.40+1.87g/dl) to 6 month of treatment (13.47+1.44g/dl) and after 28 months of treatment the value is 13.9+3.11g/dl. 12 patients were splenectomized before start of therapy. 18 patients, of whom 17 did not undergo splenectomy, had thrombocytopenia (average 68700+ 28940/mmc). After 6 months of therapy the platelet count increased at 116560+66290/mmc and up to 158420+41483/mmc after 28 months of ERT. Splenomegaly decreased from a mean of 14.512+7.87 to 4.60+3.05 of normal value for weight and hepatomegaly improved from 1.42+0,43 to 1.06 of normal value for weight.

**Conclusions:** Enzyme replacement therapy (ERT) administred for 28 months in Romanian patients with Gaucher disease type 1 led to a marked improvement in haematologic parameters and an important regression of hepato- and splenomegaly.

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