

# RESPONSE TO REPLACEMENT ENZYME TREATMENT IN PATIENTS WITH TYPE I GAUCHER'S DISEASE

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## BACKGROUND

Enzyme Replacement Therapy (ERT) is the current standard therapy which significantly improves the quality of life of patients with type 1 Gaucher Disease (GD-1).

## PURPOSE

Evaluation of the use of ERT in patients with GD-1.

## MATERIAL AND METHODS

Observational retrospective study with review of the patient's clinical history. Adult patients were included with GD-1 treated with imiglucerase or velaglucerase.

ERT response based on:

- 1) Hemoglobin ( $\geq 11-12$ g/dl)
- 2) platelets ( $\geq 120 \times 10^9/L$ )
- 3) Quitotriosidase ( $< 600$ nmol/mLxh)
- 4) liver volume ( $< 1,25$  times normal)
- 5) spleen volume ( $< 5$  times normal)
- 6) bone pain (none, mild, moderate, severe).

The type of response is established according to criteria compliance:

- 1) OR optimal response (5/6)
- 2) SR suboptimal response (3-4/6)
- 3) non  $\leq 2$ .

➤ Detection of Adverse Drug Events (ADEs).

## RESULTS

We included 3 patients (2 with imiglucerasa, 1 velaglucerase).

Previously: thrombocytopenia and/or anemia, hepatosplenomegaly and bone pathologies associated.

Dosage imiglucerase and velaglucerase:

60UI/kg/2weeks =>> reductions of 25% up to 15 IU/kg.

Median follow-up time: 7 years (5-8).

### Averages Hematologic parameters :

- Hb 14, 7(13, 2-15, 8),

-Platelets 207(174-312)

Optimal levels of quitotriosidase

Reduction of hepatic and splenic volumes

Bone pain is not resolved in all patients with bone pathologies associated (1 patient with an episode of generalized bone pain).

### Currently 2 patients with ERT:

- ❖ 2 changed to substrate reduction therapy with miglustat
- ❖ 1 patient returns to ERT therapy by intolerance.

### Clinical Response:

OR in 3 patients with 5/6 compliance criteria.

Similar safety profile and very well tolerated without unexpected ADEs:

- 1 velaglucerase (rash and diarrhea resolved)
- 1 imiglucerase (warmth infusion-related).

➤ Preparation of infusion solutions: Pharmacy

➤ Optimizing the use of vials imiglucerase: the summons of the 2 patients on the same day.

## CONCLUSION

✓ In patients with GD-1 studied, the ERT is shown effective and safe. Imiglucerase and velaglucerase presented similar responses and maintained optimal levels hematological and volume liver-spleen, with improvements in the quality of life in this patients. Patients reach and maintain the OR with good tolerance.

✓ The communication between the medical and pharmaceutical professionals is essential for an appropriate dose adjustment and optimization of the vials.

