

# Use and effectiveness of eltrombopag in a tertiary hospital

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## INTRODUCTION

Eltrombopag is authorized by the EMA for adult chronic immune thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments and as second-line treatment for non-splenectomised patients for whom surgery is contraindicated. Eltrombopag was effective in 59% of patients in a randomized controlled trial <sup>1</sup>

## OBJECTIVES

- 1) To determine whether eltrombopag is prescribed according to the approved indications.
- 2) To observe the effect on platelet levels.

## METHODS

Observational study. We included patients treated with eltrombopag from 01/01/2011 to 31/08/2011. Variables: demographics, diagnosis, previous treatments, duration, rescue medication, changes in platelet levels, and reason for suspension (where applicable).

## RESULTS

N= 7

### Previous treatments

- First line treatment: all have received corticosteroids and immunoglobulins
- Second line treatment: all were refractory to at least 2 of the follow treatments:
  - Immunosuppressants (3 patients)
  - Rituximab (3)
  - Vinca alkaloids (2)
  - Tranhexamic acid (3)
  - Romiplostim (2)

### Demographics

- Sex: 4 males; 3 females.
- Median age: 65 years old.

### Diagnosis

- ITP: 6
- Multifactorial essential thrombocytopenia: 1

### Splenectomy (patients with ITP)

- One patient splenectomised
- 5 not splenectomised:
  - Old age (3 patients)
  - Multiple comorbidities (1)
  - Refusal (1)

### Duration and effectiveness

**Continued treatment:** 3 patients  
(median interval from onset, 46 days)

3 of 3 increased platelet levels from baseline and maintained levels  $>50 \times 10^3/\mu\text{L}$

**Stopped treatment:** 4 patients  
(median duration, 87 days)

3 did not increased platelet levels

1 had uncontrolled bleeding events

Rescue with immunoglobulins needed.

## CONCLUSIONS

Eltrombopag was prescribed according to the approved indication in 6 out of 7 patients and was effective in half of the patients with ITP. Despite our small study population, the percentage of responders was similar to that found by Bussel et al.

1- Bussel J, Provan D, Shamsi T, Cheng G, Psaila B, Kovaleva L et al. *Effect of eltrombopag on platelet counts and bleeding during treatment of chronic idiopathic thrombocytopenic purpura: a randomised, double-blind, placebo-controlled trial.* Lancet 2009; 373: 641-48