

# Eltrombopag – a new treatment for thrombocytopenia

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**E**ltrombopag is a new thrombopoietin receptor agonist administered by oral route, which has been proven to increase platelet count in preclinical studies on animals and normal humans. Two recent phase II studies evaluated the effect of this new drug in the treatment of thrombocytopenia associated with hepatitis-C induced liver cirrhosis (TPL102357 trial) and in the treatment of chronic immune thrombocytopenic purpura (PTI) refractory to standard treatment (glucocorticoids and intravenous immunoglobulins).

The TPL trial included 74 patients with liver cirrhosis and moderate thrombocytopenia (20,000 – 70,000 / mm<sup>3</sup>) who were randomized to four treatment groups: placebo and active treatment with 30mg, 50mg and 75mg eltrombopag, respectively. The primary objective was the achievement of a platelet count of at least 100,000 / mm<sup>3</sup> after four weeks of treatment. This objective was achieved in a dose-dependent manner in the active treatment groups (75%, 79%, 95%, respectively) as compared with 0% in the placebo group (p<0.001). After this interval, 49 of the 74 patients included in the trial received antiviral

therapy for hepatitis C (interferon plus ribavirine) for 12 weeks in association with active or placebo study drug. The rate of completeness of the antiviral therapy in the above four groups were 6%, 36%, 53% and 65%, respectively.

The second study included 118 patients with drug-refractory PTI and severe thrombocytopenia (<30,000 / mm<sup>3</sup>). Patients were randomized in four groups, with the same doses as in the TPL study. The primary objective was the achievement of a platelet count of over 50,000 / mm<sup>3</sup> after 43 days of treatment. In the four groups of treatment (placebo and three active treatment groups), the primary objective was achieved in a dose-dependent manner: 11%, 28%, 70% and 81%, respectively. The mean platelet count in the four groups after 43 days of treatment was 16,000, 26,000, 128,000 and 183,000/ mm<sup>3</sup>, respectively. The incidence of bleeding was smaller in the active treatment group.

In conclusion, eltrombopag is a new drug that has proven efficacy in the treatment of significant thrombocytopenia associated with hepatitis-C induced liver cirrhosis and in drug-refractory PTI.

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#### Comment on the paper:

1. **McHutchison JG, Dusheiko G, Shiffman ML, et al.** – Eltrombopag for Thrombocytopenia in Patients with Cirrhosis Associated with Hepatitis C. *N Engl J Med* 2007; 357:2227-2236
2. **Bussel JB, Cheng G, Saleh MN, et al.** – Eltrombopag for the Treatment of Chronic Idiopathic Thrombocytopenic Purpura. *N Engl J Med* 2007; 357:2237-2247