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## Eltrombopag for immune thrombocytopenia secondary to chronic lymphoproliferative disorders: a phase 2 multicenter study

Carlo Visco, Francesco Rodeghiero, Alessandra Romano, Federica Valeri, Michele Merli, Giulia Quaresimini, Stefano Volpetti, Roberto M Santi, Giuseppe Carli, Elisa Lucchini, Francesco Passamonti, Alessandro Rambaldi, Giovanna Motta, Alessandra Borchiellini, Emanuele S G d'Amore, Marco Ruggeri

### Abstract

Immune thrombocytopenia (ITP) secondary to chronic lymphoproliferative disorders (LPDs) is poorly responsive to conventional treatments. We conducted a multicenter phase 2 prospective 24-week study in 18 patients with ITP secondary to LPDs to assess the safety and efficacy of eltrombopag. Responsive patients entered an extension study for up to 5 years. For inclusion, patients should not require cytotoxic treatment and should have a platelet count  $<30 \times 10^9/L$  or have symptoms of bleeding. Eltrombopag was initiated at 50 mg/day, with a maximum of 150 mg/day. The primary end point was platelet response after 4 weeks. Median age was 70 years (range, 43-83 years), and 14 patients had chronic lymphocytic leukemia, 2 had classic Hodgkin lymphoma, and 2 had Waldenström macroglobulinemia. All patients had received previous ITP treatments. Response rate at week 4 was 78% (95% confidence interval [CI], 58%-97%), with 50% of patients having a complete response (CR) (95% CI, 43%-57%); respective results at week 24 were 59% (95% CI, 36%-82%) with 30% reaching a CR (95% CI, 8%-52%). Median exposure to eltrombopag was 16 months; median dose at week 4 was 50 mg/day (range, 25-100 mg/day), and at week 24, it was 50 mg/day (range, 25-150 mg/day). No grade  $>2$  adverse events were reported. Eltrombopag is active and well tolerated in ITP secondary to LPDs. This trial was registered at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) as [#NCT01610180](https://clinicaltrials.gov/ct2/show/study/NCT01610180).