Eltrombopag second-line therapy in adult patients with primary immune thrombocytopenia in an attempt to achieve sustained remission off-treatment: results of a phase II, multicentre, prospective study

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Abstract

Up to 30% immune thrombocytopenia (ITP) patients achieve a sustained remission off-treatment (SROT) after discontinuation of thrombopoietin receptor agonists (TPO-RAs). Factors predictive of response are lacking. Patients aged ≥18 years with newly diagnosed or persistent ITP were treated with eltrombopag for 24 weeks. Primary end-point was SROT: the proportion of responders that were able to taper and discontinue eltrombopag maintaining the response during a period of observation (PO) of six months. Secondary end-points included the association between some immunological parameters (TPO serum levels, cytokines and lymphocyte subsets) and response. Fifty-one patients were evaluable. Primary end-point was achieved in 13/51 (25%) treated patients and 13/34 (38%) patients who started the tapering. Baseline TPO levels were not associated with response at week 24 nor with SROT. Higher baseline levels of IL-10, IL-4, TNF-α and osteopontin were negative factors predictive of response (P = 0.001, 0.008, 0.02 and 0.03 respectively). This study confirms that SROT is feasible for a proportion of ITP patients treated with eltrombopag. Some biological parameters were predictive of response.

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