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

Elisa Lucchini, Francesca Palandri, Stefano Volpetti, Nicola Vianelli, Giuseppe Auteri, Elena Rossi, Andrea Patriarca, Giuseppe Carli, Wilma Barcellini, Melania Celli, Ugo Consoli, Federica Valeri, Cristina Santoro, Enrico Crea, Marco Vignetti, Francesca Paoloni, Casimiro Luca Gigliotti, Elena Boggio, Umberto Dianzani, Ilaria Giardini, Monica Carpenedo, Francesco Rodeghiero, Renato Fanin, Francesco Zaja, for Gruppo Italiano Malattie Ematologiche dell'Adulto (GIMEMA)

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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