Long term response of UK children treated with Eltrombopag

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Abstract:

Background: The PETIT studies were randomised, multicentre, placebo-controlled studies which demonstrated the efficacy, tolerability and safety of Eltrombopag in the paediatric ITP population. Follow up of the patients was limited to 24 weeks in the open label phase [1,2].

Methods: 13 children from 3 UK sites were entered into the PETIT studies. Following the study period patients continued eltrombopag through a named patient protocol. On-going management was performed by local standard of care. One further child accessed Eltrombopag off study. The mean follow up of these children is now 33 months (range 7 to 69 months).

Results: All 14 children had an initial response to Eltrombopag and continued therapy for a minimum of 7 months. No hepatobilliary or thrombotic complications were reported. Five children discontinued therapy; One at 7 months due to difficulty swallowing medication, one child at 7 months due to abdominal pain and headaches and subsequently underwent splenectomy, one sub-clinical lens opacity was identified on ocular examination at 20 months and the patient was subsequently switched to Romiplostin, two patients lost response at 10 and 20 months respectively with subsequent treatment on Romiplostin and observation respectively. 3 children were able to maintain platelet counts over 100 x 10^9/L off therapy at 20, 24 and 43 months. 7 children currently continue on therapy at 36-69 months follow up. 11 children had bone marrow assessments for reticulin staining pre-therapy and on-therapy after at least 12 months. Two children showed an increase of reticulin staining by two grades and two children by one grade.

Conclusion: Ocular lens changes were reported in two patients in PETIT and emphasises the need for annual ocular review [2]. The bone marrow reticulin data is similar to that reported in adults [3]. We observed spontaneous remissions in three children and recommend weaning Eltrombopag after 24 months to assess for spontaneous improvement or remission.

References:

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