



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

311.DISORDERS OF PLATELET NUMBER OR FUNCTION: CLINICAL AND EPIDEMIOLOGICAL

Long-Term Eltrombopag in Children with Chronic Immune Thrombocytopenia: A Single-Center Extended Real-Life Observational Study in China

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Background: We have previously confirmed the efficacy and safety of eltrombopag (ELT) in children with chronic immune thrombocytopenia (cITP). However, there is still a lack of results on long-term ELT therapy. Here, we report the long-term follow-up results (between September 2018 and June 2023) on our previous study.

Methods: This is a single-center real-life observational study that collected data from patients who previously enrolled in our children cITP study and had a follow-up period of more than 3 years by June 2023.

Results:

A total of 65 patients (28 males) were enrolled with a median age at ELT initiation of 6.34 (range 1.65, 14.13) years and a 47.07 (36.00, 57.00) months follow-up time with a 40.36 (10.53, 56.83) months of ELT therapy at the analysis time point.

In total, 29.23% (19/65) (of) patients discontinued ELT because of a stable response, and 18.46% (12/65) patients switched to another platelet-enhancing therapy due to loss of response to therapy after 19.13 (14.53, 26.37) months. Among the 19 patients who discontinued ELT because of a stable response, 24.62% (16/65) children achieved 12 m of off ELT treatment (SRoT), the last recorded platelet count ranged from 56-166 × 10⁹/L (median value 107 × 10⁹/L), and 4.62% (3/65) patients relapsed at the 5th, 6th, and 9th months after discontinuation, respectively. Of the 12 patients who lost response to ELT after 19.13 (14.53, 26.37) months of therapy, four switched to avatrombopag, three to hetrombopag, two to Chinese traditional medicine, one had splenectomy and two received additional prednisolone acetate. Thirty-four patients maintained a sustained response while continuing on ELT treatment but with tapering doses. No significant adverse events were reported. In the first 12 months of ELT, there was a higher rate of PLT ≥ 100 × 10⁹/L in SRoT patients than in tapering patients (62.50% [10/16] vs. 5.88% [2/34]) and a higher rate of PLT 70-100 × 10⁹/L (31.25% [5/16] vs. 27.78% [10/36]) (P=0.01).

Conclusion:

We report more than 3 years long-term clinical data on cITP children taking ELT. We show that 24.62% of the children who had a stable response to ELT achieved 12 m of SRoT. The treatment response of children in the first 12 months after the initiation of treatment may be a predictive factor for successful discontinuation. These data highlight no new safety concerns regarding the long-term use of ELT in children with cITP.

Disclosures Poon: University of Calgary: Current Employment; KVR Pharma, Novo Nordisk, Octapharma, Sobi, Takeda: Honoraria.

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