

Use of Eltrombopag in Children with Immune Thrombocytopenia

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Eltrombopag (Promacta) is an oral thrombopoietin (TPO)-receptor agonist that has been widely used in adults with chronic immune thrombocytopenia (cITP) with good success and minimal toxicity since its initial approval in 2008.¹⁻⁴ Eltrombopag was recently approved in the US for use in children ≥ 1 year of age with cITP based on 2 large randomized, placebo-controlled trials including 171 children.⁵⁻⁶ The key points from these studies were:

1. The primary platelet response rate is high: 60%- 90% depending on the criteria used
2. The time to respond varied with the patient, the dose required to respond, and the time to steadily escalate to that dose, and may take weeks to occur. Platelet responses during the first 6 weeks of the studies are shown in Figure 1
3. As expected, increases in platelet counts were associated with reductions in bleeding and the need for rescue medications
4. Responders were often able to taper off concomitant ITP medications (such as prednisone)

Eltrombopag is generally well tolerated; across all the large studies in adults and children, approximately 3% of patients were unable to tolerate a therapeutic dose of eltrombopag. The main toxicity associated with eltrombopag is elevated aminotransferases, which is often mild and reversible. Risks associated with TPO-receptor agonists include bone marrow fibrosis, which is unlikely to be clinically significant, and thrombosis, which is observed much less frequently in children than it is in adults. Finally, there are important dietary directions to follow while administering eltrombopag; therefore, both physicians and nurses should work in partnership with the parent/caregiver to optimize management of pediatric patients and treatment with eltrombopag. In summary, eltrombopag is a highly effective medication that is taken orally every day and may help safely increase platelet counts in the majority of patients.

Biography

Katie J. Atkinson is a Family nurse practitioner in Office of Dr. James Bussel, Weill Cornell Medicine Pediatric Haematology/Oncology, Platelet Research & Treatment Program.

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