

Beyond immune thrombocytopenia: the evolving role of thrombopoietin receptor agonists

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Abstract

Since its discovery, the thrombopoietin (TPO) pathway has been an important pharmaceutical target for the treatment of thrombocytopenia. The first generation of TPO mimetics included peptide agents sharing homology with endogenous TPO, but these introduced a risk of antibody formation to endogenous TPO and were not successful. However, second-generation TPO mimetics or TPO receptor agonists (RAs) are currently being used to treat thrombocytopenia associated with a number of conditions, such as immune thrombocytopenia (ITP), severe aplastic anaemia (SAA), and hepatitis C virus-associated chronic liver disease. Accumulating efficacy and safety data suggest that the role of TPO-RAs in the treatment of thrombocytopenia may evolve in the near future with broader use of these agents in ITP and SAA, as well as approval in other indications, potentially including myelodysplastic syndromes, chemotherapy-associated thrombocytopenia, and post-transplant thrombocytopenia. This review provides an overview of clinical data on the efficacy and safety of TPO-RAs, emphasising recent findings that may expand their clinical utility.