

## Thrombopoietin receptor agonists in hereditary thrombocytopenias

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### Abstract

Hereditary thrombocytopenias (HTPs) constitute a heterogeneous group of diseases characterized by a reduction in platelet count and a potential bleeding risk. As a result of advances in diagnostic methods, HTPs are increasingly being identified, and appear to be less rare than previously thought. Most HTPs do not have effective treatments, except for platelet transfusion when bleeding occurs and in preparation for procedures associated with a risk of bleeding. Preliminary clinical evidence suggests that thrombopoietin receptor agonists (TPO-RAs) with an established use in the treatment of certain acquired thrombocytopenias are well tolerated and provide clinical benefits in patients with some forms of HTP. These drugs may therefore be considered for the treatment of HTPs in clinical practice. However, caution and close monitoring are recommended, owing to the absence of long-term safety data and the potential risks posed by prolonged bone marrow stimulation in certain HTPs. In this review, we summarize the available clinical data on TPO-RAs in the treatment of HTPs, and discuss their use in patients with these disorders. We believe that TPO-RAs will play a major role in the treatment of HTPs, particularly myosin heavy chain 9-related disease, Wiskott-Aldrich syndrome, X-linked thrombocytopenia, and thrombocytopenia caused by THPO mutations.