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Recent progress in ITP treatment

Francesco Rodeghiero

Abstract

In this review, the recently approved drugs avatrombopag and fostamatinib, which were not extensively covered within 2019 international recommendations for ITP, will be discussed in some detail. Avatrombopag appears more convenient than eltrombopag as it does not require dietary restrictions or subcutaneous administration like romiplostim. However, data on quality of life (QoL) are lacking and the rate of thromboembolic events in exposed patients is not negligible. Efficacy of fostamatinib, an inhibitor of macrophagic activity, is supported by placebo-controlled trials in patients refractory to several therapies, including TPO-RA. While hypertension and diarrhea have been reported, only one minor thrombotic event occurred in 146 exposed patients. In addition, several new treatment combinations and new agents entered clinical investigation in recent years. In a UK trial, combining mycophenolate mofetil with corticosteroids as first line therapy was more effective than corticosteroids alone, but at the cost of worse QoL. No combination, including oseltamivir or all-trans retinoic acid or danazol, resulted in convincing evidence of superior efficacy and safety when used in first or later lines of treatment. Agents targeting specific mechanisms are also discussed: sutimlimab (complement inhibitor); rilzabrutinib (BTK inhibitor) and efgartigimod (modified Fc fragment inhibiting FcRn). Only efgartigimod has completed phase 3 investigation.