portadores de deficiência hereditária de FVII dificulta a abordagem terapêutica adequada com reposição de rFVIIa. Este caso corrobora o conceito de ausência de correlação direta entre níveis plasmáticos do FVII e gravidade das manifestações clínicas, mas também levanta a discussão sobre o papel da plaquetopenia como potencial cofator hemorrágico em coagulopatias subjacentes. Convém salientar que a paciente, embora submetida a diversos desafios hemostáticos ao longo da vida, apresentou sangramento grave apenas na vigência de plaquetopenia. Por ser condição rara, a descrição de casos semelhantes pode ser útil para a melhor compreensão de potenciais preditores de risco de sangramento, auxiliando na condução individualizada desses pacientes.

https://doi.org/10.1016/j.htct.2025.104972

ID – 1960

SPLEEN TYROSINE KINASE INHIBITORS FOR IMMUNE THROMBOCYTOPENIA: A META-ANALYSIS OF RANDOMIZED CONTROLLED TRIALS

NLC Pereira [a], LOS Santos [a], FBDO Torres [a], LSUX Rodrigues [a], MV Costa [a], A Saldanha [b]

[a] *Universidade Estadual de Ciências da Saúde de Alagoas (UNCISAL), Maceio, AL, Brazil*
[b] *Hemocentro de Alagoas (HEMOAL), Maceio, AL, Brazil*

Introduction: Immune thrombocytopenia (ITP) is an autoimmune disorder, characterized by decrease of the platelet count and increased risk of bleeding. Although spleen tyrosine kinase (SYK) inhibitors have shown promise in treating ITP, no meta-analysis has yet synthesized the evidence from randomized controlled trials (RCTs) across the entire class. Therefore, there is no consolidated evaluation of the efficacy and safety of SYK inhibitors as a group, nor comparisons among different agents, limiting guidance for clinical practice and research. **Objectives:** This paper aims to synthesize the evidence from RCTs for SYK inhibitors in adults with chronic ITP and to compare the effects of different agents, conditional on the availability of data. **Material and methods:** Eligible studies were phase III RCTs comparing SYK inhibitors to placebo in ITP patients and reporting at least one relevant clinical outcome. Studies with overlapping populations, duplicate reports, or secondary ITP were excluded. Data were extracted from published reports on PubMed, Embase, and Cochrane. Risk Ratios (RR) with 95% Confidence Interval (95% CI) were pooled across trials. The primary efficacy outcome was the stable response rate (defined as ≥ 4 platelet count ≥ 50×10^9/L in 14-24 weeks). Secondary efficacy endpoints included other platelet count improvements and the need for rescue therapy. Safety outcomes comprised any Adverse Events (AEs) and other AEs. **Results:** Four trials (n = 372) were included. Syk inhibitors were given to 249 patients (66.9%): 123 (49.3%) received Fostamatinib and 126 (50.6%) Sovlepenib. SYK inhibitors significantly increased stable and overall platelet responses compared to placebo (RR = 17.77; 95% CI 5.13–61.61; $p < 0.00001$ and RR 4.17; 95% CI 2.63–6.61; $p < 0000.1$). Among individuals presenting with more pronounced thrombocytopenia at baseline (< 15×10^9/L), SYK inhibitors were favored in the increase of platelet count of ≥ 30×10^9/L and ≥ 20×10^9/L from baseline at weeks 12 and 24 (RR = 3.53; 95% CI 1.96– 6.37; $p < 0.0001$, and RR = 3.67; 95% CI 2.00–6.71; $p < 0.001$). SYK inhibitor use reduced rescue treatment need but increased AEs, particularly hypertension, while severe AEs were not significantly different. Subgroup analyses comparing different SYK inhibitors were exploratory, due to the low number of studies involved, and none of the outcomes demonstrated a statistically significant difference between subgroups. **Discussion and Conclusion:** The present study is the first systematic review with meta-analysis to specifically evaluate the class of SYK inhibitors for the treatment of chronic ITP based solely on data from RCTs. Compared to the previous meta-analysis restricted to fostamatinib, in their subgroup analysis of RCTs, our findings suggest a stronger treatment effect for SYK inhibitors overall, particularly for stable response (RR 17.77 vs. 6.43) and overall response (RR 4.17 vs. 3.04), while the direction of benefit remained consistent across studies. In conclusion, this study provides evidence supporting SYK inhibitors as viable options in the treatment of chronic ITP. Given the small number of contributing studies, these results should be interpreted cautiously. Large-scale multicenter RCTs are needed to assess the safety and effectiveness of different SYK inhibitors in order to guide clinical decisions and optimize ITP management.

https://doi.org/10.1016/j.htct.2025.104973

ID – 1562

TERAPIAS EM HEMOFILIA B: PADRÃO vs. INOVADORAS – REVISÃO DE LITERATURA SOBRE SEGURANÇA, EFICÁCIA E DESFECHOS

DM Lobert [a], CM Duarte [a], MM Mantovani [a], RO Coelho [b]

[a] *Faculdade de Medicina de Botucatu, Universidade Estadual Paulista (FMB-UNESP), Botucatu, São Paulo, Brasil*
[b] *Hospital das Clínicas da Faculdade de Medicina de Botucatu, Universidade Estadual Paulista (HCFMB-UNESP), Botucatu, São Paulo, Brasil*

Introdução: A Hemofilia B (HB) é uma coagulopatia rara, ligada ao X, que levam à produção deficiente do Fator IX (FIX). Pacientes com HB grave apresentam sangramentos espontâneos frequentes, com risco de artropatia crônica e hemorragias fatais. O tratamento padrão é a profilaxia com concentrados de FIX de meia-vida padrão (SHL). Apesar de eficazes, infusões Intravenosas (IV) frequentes comprometem adesão e controle ideal. Novas abordagens incluem FIX de meia-vida estendida (EHL), terapias gênicas (scAAV2/8-LP1-hFIXco, etranacogene dezaparvovec e fidanacogene elaparvovec) e agentes não-fatoriais (concizumab, marstacimab, fitusiran). Apesar de promissoras em reduzir a carga terapêutica