



RUXOLITINIB FOR REFRACTORY GRAFT-VERSUS-HOST DISEASE IN PAEDIATRIC PATIENTS

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BACKGROUND AND OBJECTIVE

- Ruxolitinib has shown efficacy in the treatment of steroid-refractory graft-versus-host disease (GVHD) after hematopoietic stem cell transplantation (HSCT) in adults, but the evidence in children is still scarce.
- Objective: To evaluate effectiveness and safety of ruxolitinib in paediatric patients with steroid-refractory GVHD.

MATERIALS AND METHODS

- A retrospective observational study including all patients treated with ruxolitinib in our paediatric hospital (January 2017-September 2021) was carried out.
- Variables collected from electronic medical records and pharmacy dispensing program were: age, sex, weight, previous treatments for GVHD, length of treatment, dose, treatment response, reasons for discontinuation and adverse events (AEs) related to ruxolitinib. Effectiveness was assessed by the clinical resolution of GVHD.

RESULTS 31 patients included 15 (44.1%) acute GVHD Median age: 13.5 (1-19) years 2 (1-4) previous lines of (aGVHD) Sex: 64.5% men (n=20) treatment 34 episodes Median weight: 36.9 (10-85) kg All of them had previously 19 (55.9%) chronic GVHD Median lenght of treatment: received steroids (cGVHD) 7.4 (1.4-52.3) months

11.8% (n=4) 2.5 mg/12h Weight<15 kg Weight 15-60 kg Weight 47-85 kg

EFFECTIVENESS OF RUXOLITINIB

COMPLETE RESPONSE aGVHD 86.7% (n=13) cGVHD 60.0% (n=9)

FAILURE
aGVHD 13.3% (n=2)
cGVHD 6.7% (n=1)

PARTIAL RESPONSE cGVHD 13.3% (n=2)

3 (20.0%) patients were receiving ruxolitinib at the moment of the analysis for cGVHD showing stable response (n=2) and improvement (n=1)

ADVERSE EFFECTS RELATED TO RUXOLITINIB

Increased serum alanine and aspartate aminotransferases 8.8% (n=3)

Herpes zoster infection 5.9% (n=2)

Hypertension 2.9% (n=1)

Anaemia 2.9% (n=1) 1 dose reduction due to grade 4 hepatic toxicity, that was resolved

CONCLUSIONS

In our study, ruxolitinib has shown effectiveness for refractory GHVD in most of the patients. The safety profile in our population is consistent with literature. Further studies in paediatric patients are warranted.