Ruxolitinib for steroid-refractory acute GVHD in Latin America

G. Antelo P., P. Viola, H. Miño, F. Fornillo, N. Cazap, C. Aisincharf, A. Gimenez, G. Maldonado, V. Sbrollini MD, J.I. Garcia A., J.C. Dupont, Dardo Riveros, J. Fernandez, P. Duarte MD, J. Solimano, L. Riera

CEMIC
Centro de Educación Médica e Investigaciones Clínicas "Norberto Quirno"

Introduction

Acute graft-versus-host disease (aGVHD) remains one of the most severe complications after allogeneic hematopoietic stem cell transplantation (allo-HSCT), associated with high early mortality. Corticosteroids are first-line therapy, yet <50% of patients achieve durable responses. Ruxolitinib, a selective JAK1/2 inhibitor, is approved for steroid-refractory aGVHD (SR-aGVHD), but there is scarce real-world data from Latin America

Aim

To evaluate the efficacy and safety of ruxolitinib in patients with SR-aGVHD in a Latin American tertiary center.

Objectives

1.To describe baseline demographic and transplant-related characteristics of patients with SR-aGVHD treated with ruxolitinib.
2.To determine the overall response rate (ORR) at days 28 and 56 after treatment initiation.
3.To evaluate overall survival (OS) and causes of death in this population.
4.To report adverse events related to ruxolitinib in the real-world setting.

Methods

Design: Retrospective case series.
Setting: CEMIC University Hospital, Buenos Aires, Argentina.
Population: From 99 allo-HSCT recipients (Jan 2015–Jan 2025), we included patients with SR-aGVHD after corticosteroid ± calcineurin inhibitor failure.
Variables: Donor type, GVHD grade, overall response rate (ORR) at days 28 and 56, overall survival (OS), and adverse events.
Analysis: Descriptive statistics; outcomes expressed as absolute numbers, percentages, and medians with interquartile ranges.

Characteristic	Value
Age at allo-HSCT, years (median [IQR])	53.0 (44.5–56.0)
Sex	
Male, n (%)	9 (47.4%)
Female, n (%)	10 (52.6%)
Disease	
Leukemias, n (%)	8 (42.1%)
Myelofibrosis, n (%)	3 (15.8%)
Lymphomas, n (%)	2 (10.5%)
Myeloma, n (%)	2 (10.5%)
MDS, n (%)	2 (10.5%)
Aplastic anemia, n (%)	1 (5.3%)
Donor type	
HI NR, n (%)	8 (42.1%)
HAPLO, n (%)	6 (31.6%)
HI REL, n (%)	4 (21.1%)
NR mismatch, n (%)	1 (5.3%)

Results

N = 19 patients: GVHD grade: III **n=9**, IV **n=10**.
Organ involvement at diagnosis: Skin in 17 patients (89.5%), gastrointestinal tract in 19 (100%), and liver in 7 (36.8%).
Day 28 ORR: 63.1% (CR 7, PR 5).
Day 56 ORR: 63.1% (CR 11, PR 1).
Median OS (from allo-HSCT, CN): 9.7 months (IQR **2.6–21.8**).
*(Median follow-up among survivors: **23.8 months**; IQR **18.6–39.3**.)*
Deaths: 12/19 (63.2%) — relapse **n=3**, infection **n=5** (sepsis/CMV/other), GVHD progression/related **n=4**.

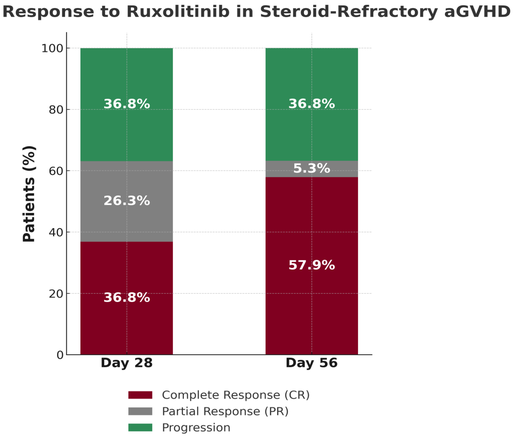


Figure 1. *Overall response rate to ruxolitinib at day 28 and day 56 in patients with steroid-refractory acute graft-versus-host disease.* Bars represent the proportion of patients achieving complete response (CR), partial response (PR), or showing disease progression at each time point. At day 28, 36.8% achieved CR, 26.3% PR, and 36.8% had progression. At day 56, 57.9% achieved CR, 5.3% PR, and 36.8% had progression.

Summary

In our Latin American real-world cohort, ruxolitinib achieved a 63.1% ORR at day 56 in SR-aGVHD, including high rates of complete response. Despite these results, mortality remained high, reflecting the aggressive nature of SR-aGVHD.

Discussion

Our results align with pivotal trials and international real-world series, reinforcing ruxolitinib as an effective option for SR-aGVHD. The absence of thrombocytopenia in our cohort may reflect careful patient selection and dose adjustments. The mortality rate emphasizes the need for early initiation and multidisciplinary supportive care. Given the limited data from the region, these findings highlight the feasibility of implementing advanced GVHD therapies in resource-constrained settings, and underscore the value of regional collaboration for optimizing post-transplant care.

References

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