REAL-WORLD EXPERIENCE WITH VENETOCLAX AS PART OF THE CONDITIONING REGIMEN FOR HSCT IN PATIENTS WITH HIGH-RISK AML/MDS

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Introduction

Patients with AML and HR-MDS often require allogeneic hematopoietic stem cell transplantation (allo-HSCT) for potential cure; however, relapse rates remain as high as 40%. The addition of venetoclax, a BCL-2 inhibitor, to conditioning regimens is currently under investigation.

Aim

This case series describes early clinical experience with venetoclax-based conditioning in AML and HR-MDS patients at a Latin American center.

Objective

To describe the clinical outcomes of AML and HR-MDS patients receiving venetoclax as part of a conditioning regimen for allo-HSCT.

Methods

This retrospective case series included AML and HR-MDS patients who underwent allo-HSCT at CEMIC University Hospital, Argentina, between May 2023 and April 2025. All patients received venetoclax 200 mg/day from day –8 to –2, combined with fludarabine and melphalan, as part of a RIC regimen.

Results

Characteristics N= 9	Value (%)
Age	45 (RIC 36-60)
Sex	
Female	4 (44%)
Male	5 (56%)
Disease	
AML (High Risk)	8 (89%)
MDS (High Risk)	1 (11%)
Donor Type	
Matched Related Donor (MRD)	3 (33%)
Matched Unrelated Donor (MUD)	3 (33%)
Haploidentical	3 (33%)
Stem Cell Source	
Bone Marrow (BM)	1 (11%)
Peripherical Blood (PB)	8 (89%)
MRD at Trasplant	
Negative	9 (100%)

Results	Value n (%)
Median days to neutrophil engrafment	19 (RIQ 16-23.5)
Median days to platelet engrafment	21.5 (RIQ 14-28)
GVDH prevalence and grades	
G1 skin	3 (33%)
G4 gastrointestinal	1 (11%)
Complications	
Mucositis	9 (100%)
Febril neutropenia	8 (89%)
CRS G1	3 (33%)
Catheter-associated DVT	1 (11%)
VOD G2	1 (11%)
Outcomes at End of Follow up	128 days (53-554)
CR	8 (89%)
MRD-negative	8 (89%)
100% donor chimerism	8 (89%)
Molecular/Morphological relapse	0
Deaths	1

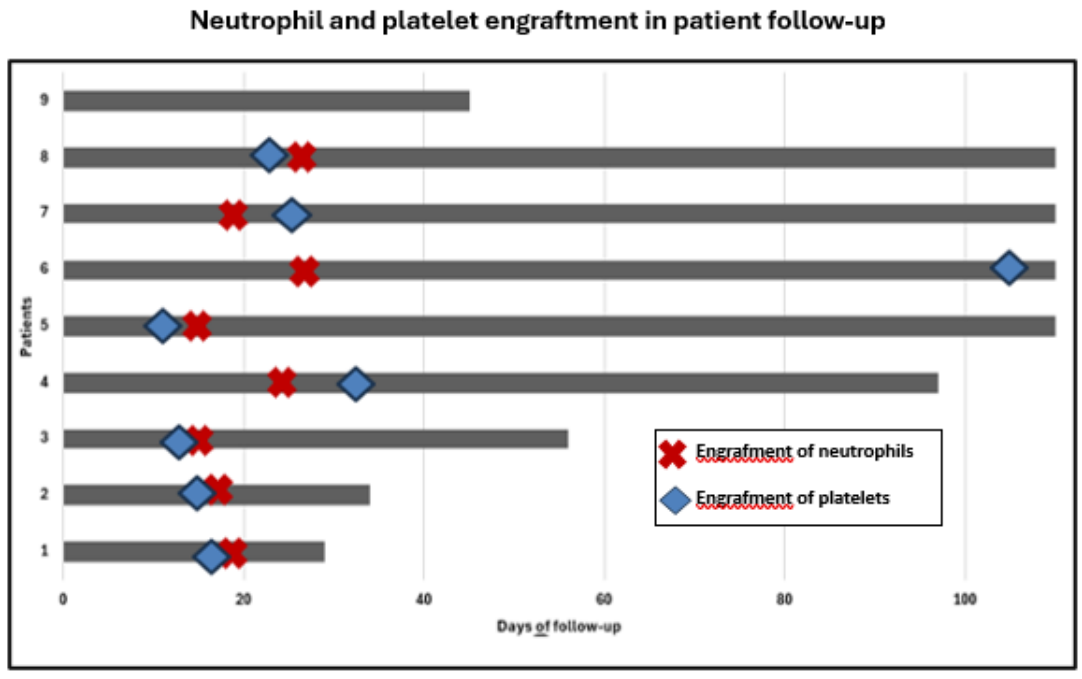


Fig 1. **Neutrophil and platelet engraftment.** The graph displays the engraftment days for each cell line during the initial follow-up period. Patient number 9 experienced primary graft failure

Conclusions

Venetoclax appears to be a safe addition to RIC regimens in AML and HR-MDS, showing promising early outcomes. Larger prospective studies are needed to confirm its safety and efficacy, particularly in real-world, resource-limited settings

References

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