ALLOGENEIC TRANSPLANT OUTCOMES IN MYELOFIBROSIS IN THE ERA OF NOVEL JAK INHIBITORS: A SINGLE INSTITUTION EXPERIENCE

G. Medawar, MD[1], Y. Perez-Melendez, MD[1], J. Marini, PharmD[2], M. McGann, PharmD[2], A. Coltoff, MD[1], P. Baratam, MBBS[1]

[1] Department of Hematology-Oncology, Medical University of South Carolina, Charleston, SC, USA

[2] Department of Pharmacy, Medical University of South Carolina, Charleston, SC, USA

MUSC Medical University of South Carolina

Introduction

Allogeneic hematopoietic stem cell transplantation (Allo-SCT) remains the only curative therapy for myelofibrosis (MF). Outcomes in the era of novel JAK inhibitors (JAKi) are not well defined.

Integration of JAK inhibitors into pre-transplant management and the use of reduced-intensity conditioning (RIC) regimens have expanded transplant eligibility.

Real-world data on efficacy and safety remain limited.

Objectives

Describe the outcomes of Allo-SCT in patients with MF at the Medical University of South Carolina (MUSC) in the contemporary era of novel JAK inhibitors

Methods

We retrospectively analyzed 11 consecutive MF patients who underwent Allo-SCT at our institution from 2020 to 2024.

Outcomes assessed included engraftment, graft-versus-host disease (GVHD), transfusion requirements, molecular remission, symptom control, and survival at a median follow-up of 15 months.

Results

Table 1. Baseline Characteristics

Patients n (%)	
Total number	11 (100)
Median age	66 (48-72)
Gender	
Males	6 (54.5)
Females	5 (45.5)
MIPSS70-plus v2	
Low	1 (9.1)
Intermediate	2 (18.2)
High	6 (54.5)
Very High	2 (18.2)
BM fibrosis grade	
Grade 1	2 (18.2)
Grade ≥2	10 (90.9)
Pre-Allo-SCT JAKi used (any line)	
None	1
Ruxolutinib	9
Fedratinib	1
Pacritinib	3
Momelotinib	2
≥2 JAKi used pre-allo-SCT	
Yes	4 (36.4)
No	8 (72.7)
Spleen irradiation	
Yes	3 (27.3)
No	9 (81.8)

Pre-transplant treatment included JAKi in 91% (predominantly ruxolitinib), with tapering strategies individualized and cessation at a median of 6 days before transplant. Only 1 patient had ruxolitinib-withdrawal syndrome.

Table 2. SCT Characteristics

Donor Type (%)	
MUD	9 (81.8)
Haploidentical	2 (18.9)
Median cell dose (CD34/kg) x10⁶	
	8.06
Stem Cell Source (%)	
	Peripheral Blood (100)
Conditioning Regimen (%)	
Myeloablative	2 (18.9)
Reduced Intensity	9 (81.8)
GVHD prophylaxis (%)	
PtCy/Tac/MMF	8 (81.8)
Tac/MMF	2 (18.9)

Neutrophil engraftment achieved in all patients, with a median time of 18 days.

Acute GVHD in 40% (4/10 evaluable), limited to grade 1–2 skin or GI involvement. Chronic GVHD in 50% (5/10), with one case of severe grade, respectively.

Marrow fibrosis cleared in 66.7% at 6 months. By 1 year, 100% (7/7) achieved full donor chimerism and clearance of JAK2/CALR mutations and other co-mutations.

At last follow-up, all evaluable patients achieved transfusion independence. No patient experienced leukemic progression/relapses, symptomatic splenomegaly (with spleen size normalization in 50%), or constitutional symptoms. No reported deaths.

Table 3. Results

	Pre-Allo-SCT	Day+100 post-Allo-SCT	6m-Post-Allo-SCT
Median CBC indices			
WBC (K/cumm)	6.5	3.4	4.8
Hgb (g/dL)	7.1	9.9	11.8
PLT (K/cumm)	141	82	93
Constitutional symptoms			
Present	1 (9.1)	0 (0)	N/A
Splenomegaly			
Nb of evaluated patients (n)	10	9	N/A
Present (% evaluable)	9 (90)	4 (44.4)	N/A
Median spleen length below the LCM (cm)			
Nb of evaluated patients (n)	11	9	N/A
Length (cm)	3	2.7	N/A
Marrow Fibrosis			
Reported (n)	11	10	7
Present (% evaluable)	11 (100)	5 (50)	2 (28.6)
Highest grade if present (%)	3 (63.6)	3 (10)	2 (14.3)
Median grade if present	3	2	0

Conclusion

Allo-SCT following JAK inhibitor therapy yielded excellent molecular remission, transfusion independence, and survival with no leukemic progression. RIC regimens and individualized JAK-inhibitor tapering enabled safe transplantation with manageable GVHD. Further studies are warranted to refine patient selection and optimize peri-transplant management.

Contact information: Georgio Medawar, MD, Medawar@musc.edu
Praneeth Baratam, MBBS, Baratamp@musc.edu