SOHO ANNUAL MEETING 2024

Allogeneic Stem Cell Transplant in Myelofibrosis Patients: A Real-World Analysis

Serena Tharakan,[1] Dahniel Sastow[1], Grace van Hyfte[2], John Mascarenhas[3], Alla Keyzner[3], Douglas Tremblay[3]

[1]Department of Internal Medicine, Icahn School of Medicine at Mount Sinai, New York, NY
[2]Institute for HealthCare Delivery Science, Department of Population Health Science and Policy, Icahn School of Medicine at Mount Sinai, New York, NY
[3]Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai, New York, NY

Mount Sinai

BACKGROUND

- Allogeneic stem cell transplant is the only curative option for myelofibrosis (MF) but many patients are ineligible due to age and comorbidities
- Even among eligible MF patients, many patients do not proceed to transplant though the reasons behind this are unclear
- Some proposed reasons for under-transplantation include referral delay, patient hesitation, and donor availability
- Unfortunately, there is a paucity of studies to analyze these potential barriers to transplant

AIM

- We aimed to better understand referral patterns and potential barriers to transplant in MF patients
- We hypothesized that referral delays and donor unavailability reduce the likelihood of successful transplant in MF

METHODS

- Retrospective analysis of 338 myelofibrosis patients diagnosed between 1985 and 2022
- Single academic center
- Transplant eligible patients: age ≤ 75, ECOG performance status <3, and DIPSS score intermediate-1 or higher
- Primary outcomes: referral to transplant and receipt of transplant

RESULTS

Table 1: Baseline patient characteristics

	Patients who were not referred to transplant (n=194)	Patients referred but did not proceed to transplant (n=80)	Transplanted patients (n=64)	p-value
Age at diagnosis *	68 (12)	60 (10)	55 (10)	(<0.001)
Gender				0.624
Male	98 (52%)	44 (56%)	36 (58%)	
Female	91 (48%)	34 (44%)	26 (42%)	
Time from diagnosis to referral (m)	--	22 (26)	36 (56)	0.212
ECOG performance status				0.07
0	62 (37%)	43 (55%)	32 (53%)	
1	82 (49%)	28 (36%)	25 (42%)	
2	19 (11%)	5 (6%)	3 (5%)	
3	5 (3%)	2 (3%)	0 (0%)	
Type of myelofibrosis				0.9548
Primary	88 (45%)	40 (50%)	29 (45%)	
Secondary	106 (55%)	40 (50%)	35 (55%)	
DIPSS score				0.369
Low risk	21 (11%)	14 (18%)	12 (19%)	
Intermediate risk 1	75 (39%)	27 (34%)	23 (36%)	
Intermediate risk 2	77 (40%)	32 (40%)	27 (42%)	
High risk	20 (10%)	7 (9%)	2 (3%)	
Splenomegaly				0.018
Yes	113 (61%)	61 (78%)	46 (72%)	
No	72 (39%)	17 (22%)	18 (28%)	
Peripheral Blood Blast Percentage *	1.1 (3.0)	0.95 (1.6)	3.1 (4.4)	<0.001
Leukemic Transformation				<0.001
Yes	13 (6.7%)	9 (11%)	17 (27%)	
No	181 (93%)	71 (89%)	47 (73%)	
Time from diagnosis to transformation+ (m)	36 (45)	35 (21)	52 (85)	0.497
Available donor				<0.001
Yes	--	29 (57%)	64 (100%)	
No		22 (43%)		
Related donor	--			0.072
Yes		19 (66%)	27 (43%)	
No		10 (34%)	36 (78%)	

Results report as mean (standard deviation)

Figure 1: Overall survival for transplanted vs non-transplanted patients

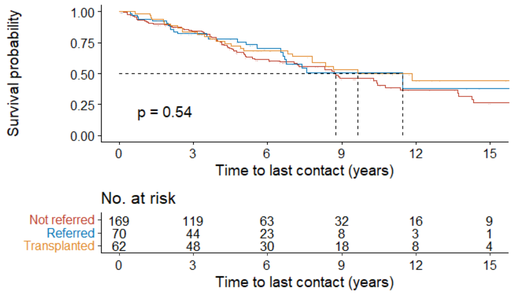


Figure 2: Factors associated with referral to transplant

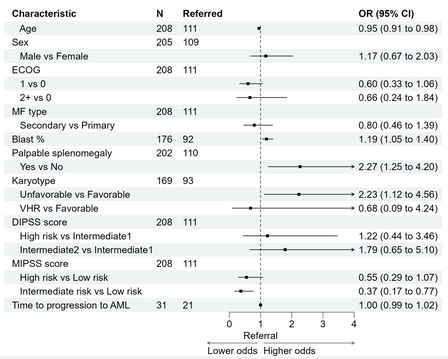
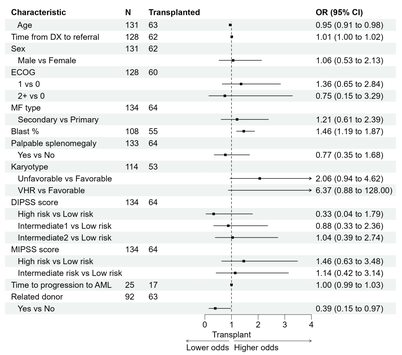


Figure 3: Factors associated with receiving transplant



DISCUSSION

- In this analysis from a single-center, we see that MF patients are likely being under-transplanted
  - 144/338 were referred (43%)
  - 64/338 were transplanted (19%)
- Time to referral did not impact likelihood of transplant
- Factors impacting referral (splenomegaly, peripheral blast percentage) likely reflect advanced nature of disease
- Patients with related donors less likely to proceed to transplant than those with unrelated donors
- Study limited by study size (only 64 transplanted patients)

CONCLUSIONS

- MF patients are slightly under-referred and largely under-transplanted
- Patients with splenomegaly were more likely be referred to transplant while patients with higher peripheral blast % were more likely to actually receive a transplant
- Donor type impacts transplant likelihood
- Future work can analyze impact of patient hesitation/attitudes towards transplant

REFERENCES

1. Rebecca Devlin, Vikas Gupta; Myelofibrosis: to transplant or not to transplant?. *Hematology Am Soc Hematol Educ Program* 2016; 2016 (1): 543–551.
2. Patriarca F, Bacigalupo A, Sperotto A, Isola M, Soldano F, Bruno B, van Lint MT, Iori AP, Santarone S, Porretto F, Pioltelli P, Visani G, Iacopino P, Fanin R, Bosi A; GITMO. Allogeneic hematopoietic stem cell transplantation in myelofibrosis: the 20-year experience of the Gruppo Italiano Trapianto di Midollo Osseo (GITMO). Haematologica. 2008 Oct;93(10):1514-22.
3. Vikas Gupta, Parameswaran Hari, Ronald Hoffman; Allogeneic hematopoietic cell transplantation for myelofibrosis in the era of JAK inhibitors. *Blood* 2012; 120 (7): 1367–1379.