

CLINICAL PROFILE, MOLECULAR LANDSCAPE, AND OUTCOMES OF MYELOFIBROSIS IN LEBANON: REAL-WORLD DATA

Hiba SMAILY¹, Hiam FAKHREDDINE¹, Nour MOUKALLED¹, Jean EL CHEIKH¹, Ali BAZARBACHI¹ and Iman ABOU DALLE^{1,*}

1. Division of Hematology/Oncology, Department of Internal Medicine, American University of Beirut Medical Center, Beirut, Lebanon

*Corresponding author



AMERICAN UNIVERSITY OF BEIRUT MEDICAL CENTER

المركز الطبي في الجامعة الأميركية في بيروت

INTRODUCTION

- Myelofibrosis (MF) is a rare chronic myeloproliferative neoplasm with variable clinical presentation, heterogeneous molecular profiles and a substantial symptom burden.
- Most existing knowledge derives from North American and European cohorts, with limited data from the Middle East.
- To address this gap and the growing need for specialised care, a dedicated MPN clinic was established at our center, prompting evaluation of patient profiles and outcomes.

AIM

- To describe the clinical, molecular and treatment characteristics of MF patients managed through a dedicated MPN clinic in Lebanon.
- To evaluate treatment patterns, patient-reported experiences and clinical outcomes in a real-world setting.
- To assess the impact of the economic crisis on treatment availability, adherence and continuity.

METHODS

- Design:** retrospective electronic medical record analysis combined with a survey-based sub-study.
- Patients:** adults diagnosed with primary or secondary MF between December 2018 and December 2025.
- Data:** clinical, molecular, laboratory, treatment and outcome variables; risk stratified by IPSS. Analysed in SPSS v26.
- Sub-study:** a subset completed a validated questionnaire on quality of life, treatment adherence and availability, and the impact of the economic crisis.
- Analysis:** Kaplan–Meier method for survival.

CONCLUSIONS

- Clinical and molecular features of MF in this cohort were broadly consistent with published data.
- Real-world differences in treatment delivery and continuity were evident, driven largely by financial and access-related barriers.
- These findings highlight the value of dedicated MPN programmes and the importance of regional data to understand the biological and healthcare-system factors influencing MF outcomes.

RESULTS

Cohort and disease

- 117 patients; median age 58 years (range 18–88)
- Primary MF 79.5%; secondary MF 20.5%
- IPSS high risk 51.8% (n = 114)

Molecular landscape

- JAK2 V617F 50.4%; CALR 10.3%
- TET2 15.4%; ASXL1 7.7%
- High molecular risk mutation in 14.5%

Treatment

- Ruxolitinib 57.8% any line, 45.1% first line
- Hydroxyurea 26.7%; allogeneic SCT 6.8%
- Transplant rate similar by risk group (p = .463)

Outcomes

- Median follow-up 47.9 months
- 5-year overall survival 89.7%
- Death 21.1% vs 1.8% and AML 17.5% vs 5.3% in high vs low risk (p = .001, p = .039)

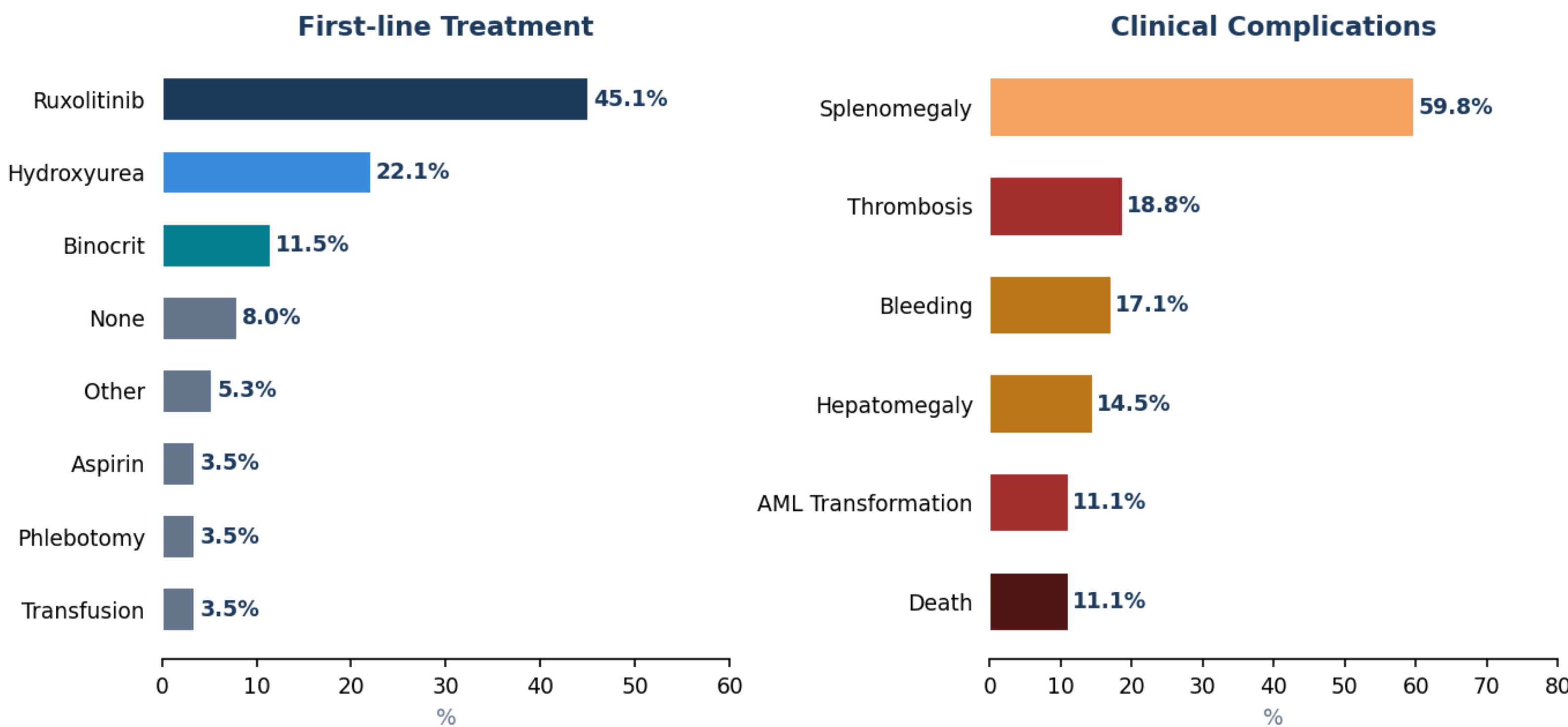


Figure 1. First-line treatment and clinical complications (N = 117).

Table 1. Clinical, molecular, treatment and outcome characteristics.

Characteristic	Value	Characteristic	Value
Patients, n	117	NGS performed:	56 (47.9%)
Age at diagnosis, median (range)	58 (18–88)	JAK2 V617F	59 (50.4%)
Male	71 (60.7%)	CALR exon 9	12 (10.3%)
Lebanese	98 (83.8%)	TET2	18 (15.4%)
Primary MF	93 (79.5%)	ASXL1	9 (7.7%)
Secondary MF (post-PV / post-ET)	24 (20.5%)	High molecular risk ‡	17 (14.5%)
IPSS/DIPSS score:		Treatment:	
IPSS high risk †	59 (51.8%)	Ruxolitinib (any line)	67 (57.8%)
IPSS intermediate †	43 (37.7%)	Hydroxyurea (any line)	31 (26.7%)
Hemoglobin < 10 g/dL	57 (48.7%)	Allogeneic SCT	8 (6.8%)
Platelets < 50 × 10 ⁹ /L	37 (31.6%)	Splenomegaly	70 (59.8%)
Normal karyotype	56 (47.9%)	Thrombosis	22 (18.8%)
Complex karyotype	4 (3.4%)	AML transformation	13 (11.1%)
Charlson index, severe risk	22 (18.8%)	Death	13 (11.1%)

n (% of N = 117). † Of the 114 patients with an evaluable IPSS score. ‡ ASXL1, EZH2, IDH1/IDH2, TP53, SRSF2 or U2AF1. Driver mutations were assessed across the cohort by targeted testing; the extended NGS panel was performed in 56 (47.9%), and non-driver mutations were detected only within that subgroup.

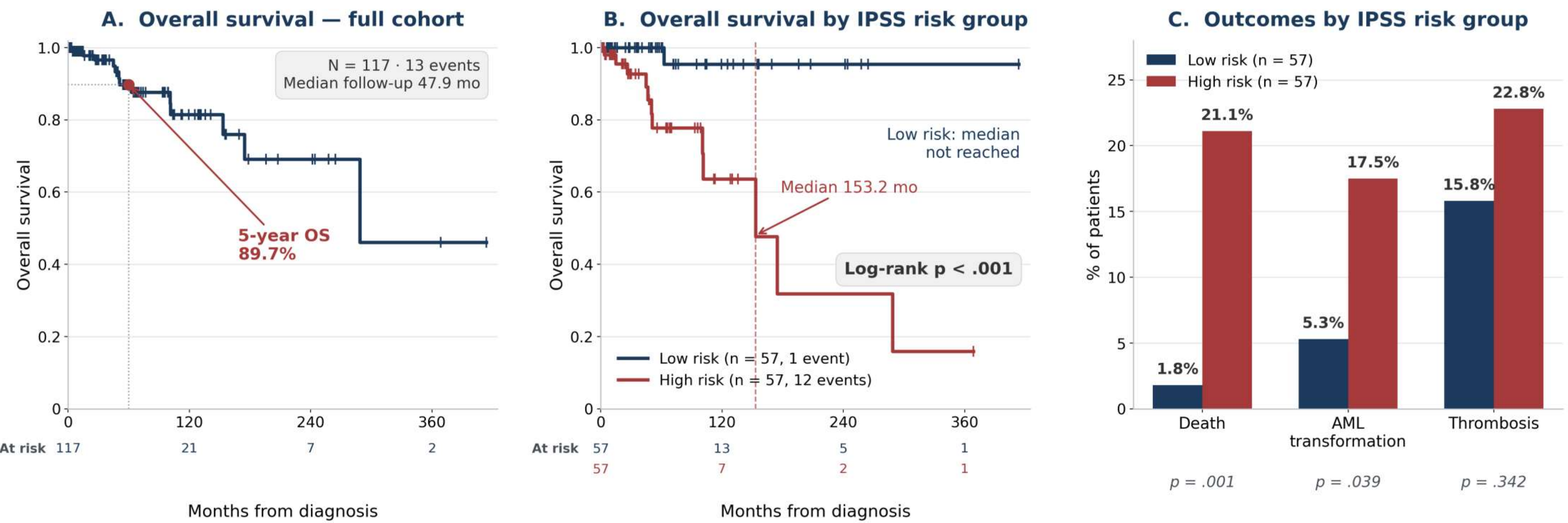


Figure 2. Overall survival for the full cohort (A), by IPSS risk group (B), and outcomes by IPSS risk group (C). Median follow-up 47.9 months by reverse Kaplan–Meier.

ACKNOWLEDGEMENTS

The authors thank the patients and their families, and the multidisciplinary team of the MPN clinic and the Bone Marrow Transplantation Program at the American University of Beirut Medical Center.

CONTACT

Hiba Smaily, MD
Division of Hematology/Oncology, Department of Internal Medicine
American University of Beirut Medical Center, Beirut, Lebanon
Email: hs162@aub.edu.lb

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