Illustrating the Societal Value of Ruxolitinib in Myelofibrosis Care in Malaysia

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INTRODUCTION

- Myelofibrosis (MF) is a chronic myeloproliferative neoplasm with substantial clinical burden. In Malaysia, the lack of a national registry and limited local data restrict understanding of disease burden, treatment patterns, and outcomes.
- Many patients present with intermediate- to high-risk disease, while gaps in diagnosis, treatment access, and healthcare prioritisation create significant unmet needs. Current therapies mainly provide symptomatic relief with limited impact on disease progression.
- **This study evaluates the clinical, humanistic, economic, and healthcare system burden of MF in Malaysia**, examines the role and value of ruxolitinib in improving patient outcomes, and explores the potential benefits of earlier access to support evidence-based policy and patient-centred care.

RESULTS

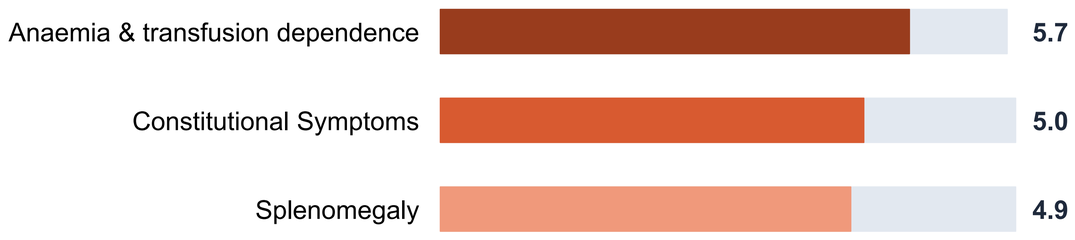
Clinical Patterns in the Malaysian Public Sector

Symptomatic MF patients often experience progressive splenic symptoms, worsening anaemia, and functional decline before treatment initiation, reflecting the disease's layered clinical and healthcare burden.

Pattern	Description
Late-stage presentation	Advanced disease on arrival; already transfusion-dependent
Administrative access barriers	Blocked by quotas/gatekeeping — not clinical reasons
Prioritisation disconnect	CML/AML funded first; chronic MF burden ignored
Clinical inertia	Delays worsen symptoms; transfusion dependence deepens
Functional decline	Work capacity lost; median survival only 5–7 years

Burden of myelofibrosis in Malaysia

- **Myelofibrosis imposes a substantial QoL burden in Malaysia, particularly among late presenters.** On a 7-point scale (1 being not at all important and 7 being extremely important), anaemia was rated as the most burdensome symptom (5.7), followed by constitutional symptoms (5.0) and splenomegaly (4.9), with clinicians highlighting the compounded impact of fatigue, frequent transfusions, hospital visits, and progressive functional decline on patients' daily lives
- **Beyond physical symptoms, MF carries major psychosocial and healthcare-system burdens.** Massive splenomegaly, drenching night sweats, fever, and weight loss severely disrupt sleep, nutrition, and social functioning, while anxiety, depression, and social isolation remain underrecognized.



Access Constraints

Although ruxolitinib is listed in Malaysia's Ministry of Health Medicines Formulary, its utilisation in the public sector is constrained by a combination of institutional and operational factors, as reported by the expert panel:

Access Constraint	Operational Manifestation	Clinical Consequence
Budget-constrained authorisation	Centre-level quotas; annual budget caps per institution rather than per eligible patient	Clinically eligible patients delayed or denied treatment based on budget cycle position, not clinical need
Funding prioritisation for acute malignancies	Chemotherapy for CML and acute leukaemia funded first within haematology budgets	Chronic progressive diseases like MF systematically queued behind conditions perceived as more acutely life-threatening

CONCLUSION

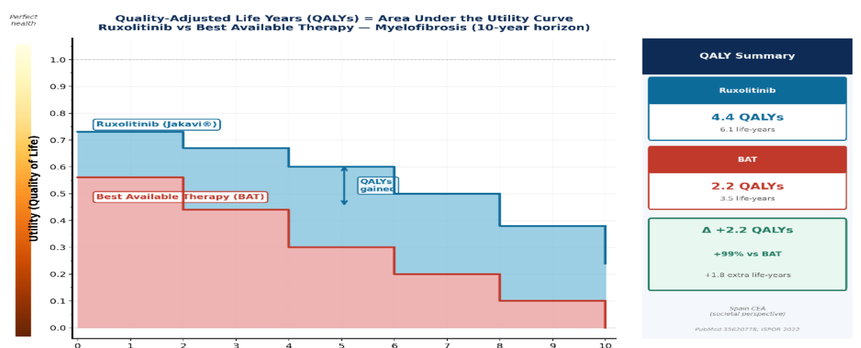
- Myelofibrosis remains under-recognised and under-prioritised in Malaysia despite its substantial clinical, humanistic, and economic burden.
- Delayed access to ruxolitinib may worsen outcomes and shift costs to transfusions, hospitalisations, caregiver burden, and productivity loss.
- Ruxolitinib provides meaningful improvements in symptom control, spleen reduction, quality of life, and survival in intermediate-2 and high-risk MF. A managed access approach with defined eligibility, designated centres, and outcomes monitoring offers a balanced and sustainable policy pathway.
- Earlier access pathways and a national MF registry are critical to support equitable, evidence-based, and sustainable MF care in Malaysia.

METHOD

- The Malaysian disease context, burden, and unmet needs were characterised through a 3-hour advisory board with five haematologists and two pharmacists from the public healthcare sector.
- A cost-utility analysis comparing ruxolitinib with best available therapy (BAT) was reviewed from a societal perspective, incorporating Malaysian healthcare costs, local clinical inputs, resource utilisation assumptions, and patient outcome considerations.
- International policy, funding, and reimbursement approaches for MF therapies were examined across selected healthcare systems to identify relevant access models and best practices that could be adapted and applied within the Malaysian healthcare setting.

Economic Value of Ruxolitinib in MF

- Cost-utility analysis compared ruxolitinib vs best available therapy (BAT) in Malaysia using a hybrid decision-tree/Markov model over 15 years
- Societal perspective captured direct medical, non-medical, and productivity-loss costs, recognising the substantial hidden burden of myelofibrosis (MF)
- BAT reflected Malaysian public-sector practice; clinical and cost inputs were based on COMFORT studies, local expert opinion, MOH fee schedules, and labour statistics
- Ruxolitinib improved survival and quality of life, generating positive incremental QALYs versus BAT
- The ICER was within Malaysia's WHO-aligned 1–3× GDP per capita threshold, supporting reimbursement value for ruxolitinib in MF



Country / context	ICER (× GDP per capita)	Reimbursement Decision
Canada	$61,444/QALY (~1.5–2.0×)	Reimbursed (provincial variation)
Portugal	€40,000/LYG (~1.2–2.2×)	Reimbursed
UK	£31,229/QALY (~1.2–2.0×)	Reimbursed via Cancer Drugs Fund
Malaysia (modelled)	RM 90,100/QALY Within 1–3× (WHO-aligned threshold)	Listed on the MOH Medicines Formulary, but with limited access

Societal Value of Ruxolitinib in MF[3,4]

✓ Reduces caregiver and family burden by decreasing reliance on recurrent transfusion-related healthcare visits, which can require substantial time commitment from accompanying family members

✓ Preserves patients' ability to work and maintain daily activities by reducing disease-related symptom burden and transfusion dependence

✓ Prevents progressive loss of functional independence — addressing the layered burden of stamina loss, appetite decline, and eventual dependency on caregivers

✓ Mitigates productivity losses at the household level by reducing the combined impact of patient work impairment and informal caregiving demands on families

✓ Shifts cost burden away from high-HCRU supportive care — earlier access reduces reliance on transfusions, hospitalizations, and emergency presentations

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