Impact of anaemia and transfusion requirement on healthcare resource utilisation in patients with myelofibrosis (MF) in France

Digital poster

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Background

- MF is a rare disease typically caused by genetic mutations in bone marrow stem cells and often results in splenomegaly, constitutional symptoms and severe anaemia.[1–3]
- JAKi are the current standard of care for systemic therapy; however, their use may also exacerbate anaemia.[4,5]
- Moderate and severe anaemia, and the often-corresponding need for RBCT, are associated with leukaemic transformation and worse survival outcomes.[6,7]
- Approximately 40% of patients with MF are anaemic at diagnosis, although nearly all patients with long enough survival become anaemic over the course of disease; almost half require transfusion therapy within 1 year of diagnosis.[8–10]
- There are currently no available data on the HCRU and cost of transfusion-requiring MF in France.

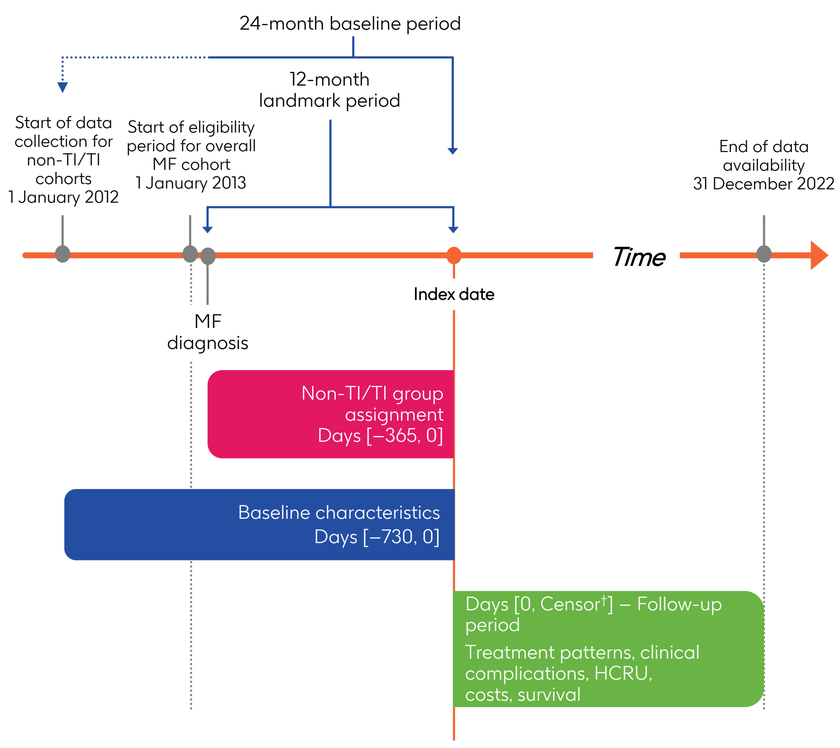
Aims

This study examined the impact of RBCT on HCRU and associated costs for patients with MF in France.

Methods

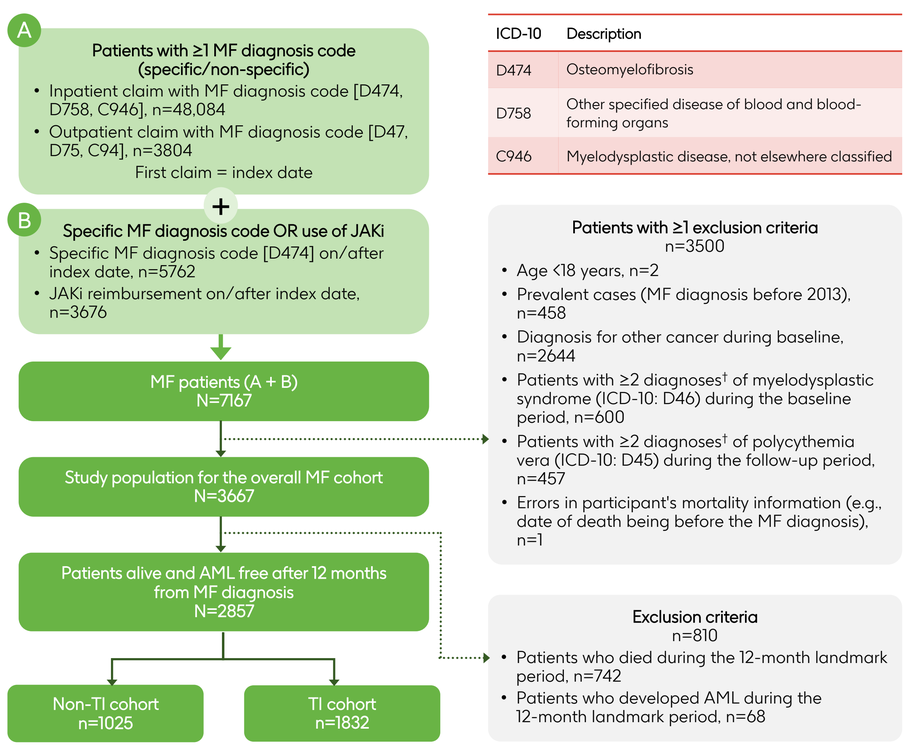
- This retrospective claims study using the French SNDS database identified patients diagnosed with MF between 01/01/2013 and 31/12/2021.
- Data collection began during the baseline period, 24 months prior to the MF index date, and continued during the follow-up period from index date to death, end of data or loss of follow-up (**Figure 1**).
  - There was a minimum of 12 months follow-up after MF diagnosis.
- All analyses were performed in two subgroups defined by patient transfusion need during the 12-month landmark period following MF diagnosis (subgroup assignment was not subject to change after the landmark period) (**Figure 1**):
  1. Non-TI (≥1 transfusion) or
  2. TI (no transfusions).
- Baseline demographics and clinical characteristics were recorded during the baseline period, and treatment utilisation, HCRU, costs and clinical outcomes were recorded during the follow-up period (**Figure 1**).

Figure 1: Overall study schema for the non-TI and TI cohorts



†Patients were censored at death (earliest death date in the linked records), last known record for the patient in the French SNDS datasets, or 31 December 2022 (end of latest data cut), whichever came first.

Figure 2: Cohort selection and attrition



ICD-10	Description
D474	Osteomyelofibrosis
D758	Other specified disease of blood and blood-forming organs
C946	Myelodysplastic disease, not elsewhere classified

†Two diagnoses at least 28 days apart.

Results

Baseline characteristics

- Out of the 2857 patients with MF who had survived and not developed AML during the landmark period, 1025 were non-TI and 1832 were TI (**Figure 2**).
- The non-TI cohort had a higher mean age (72.6 vs 66.9 years), a greater proportion of males (59.7% vs 53.8%), more patients with secondary MF (60.6% vs 34.7%) and more comorbidities (mean CCI: 2.0 vs 1.3) compared with the TI cohort (**Table 1**).

Table 1: MF patient demographics and clinical characteristics for non-TI and TI cohorts

	Non-TI cohort N=1025	TI cohort N=1832
Sex, n (%)		
Male	612 (59.7)	985 (53.8)
Female	413 (40.3)	847 (46.2)
Age at index date (years)		
Mean (SD)	72.6 (12.1)	66.9 (14.9)
Median (IQR)	74.0 (66.0–81.0)	69.0 (58.5–78.0)
<65, n (%)	218 (21.3)	675 (36.8)
65–74, n (%)	309 (30.1)	565 (30.8)
75+, n (%)	498 (48.6)	592 (32.3)
Clinical characteristics, n (%)		
Thrombocytopenia	276 (26.9)	156 (8.5)
Secondary MF	621 (60.6)	636 (34.7)
Anaemia†	1025 (100)	577 (31.5)
CCI		
Mean (SD)	2.03 (2.2)	1.33 (1.9)
Median (IQR)	1 (0–3)	1 (0–2)
CCI = 0, n (%)	302 (29.5)	862 (47.1)
CCI = 1, n (%)	211 (20.6)	412 (22.5)
CCI ≥2, n (%)	512 (50.0)	558 (30.5)

†Claim with MF-related anaemia diagnosis, reimbursement for ESAs or danazol or RBCT during the baseline period.

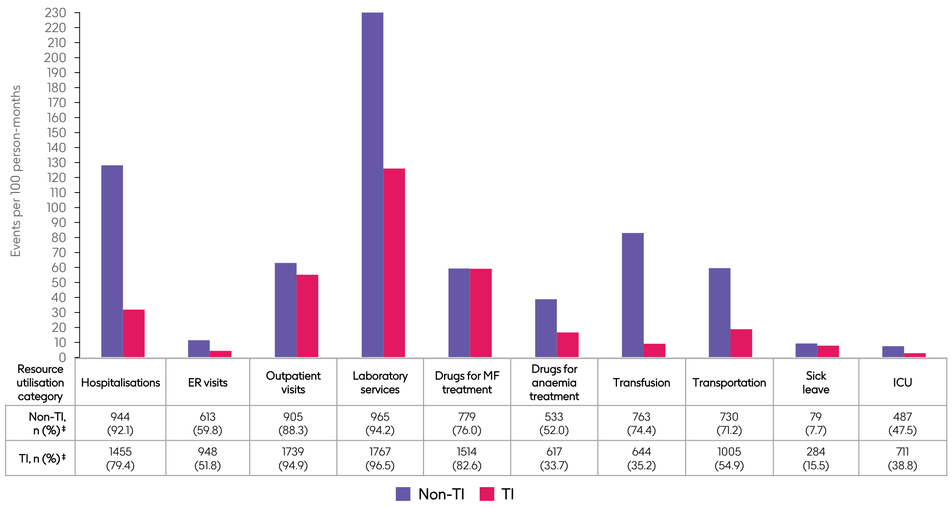
Clinical outcomes and treatment patterns

- Among the non-TI cohort, the mean transfusion rate was 1.14 PPPM during a mean follow-up of 2.1 years versus 0.26 transfusions PPPM during a mean 3.6-year follow-up in the TI cohort.
- The difference in follow-up duration was likely driven by differences in survival (median time to death: non-TI, 2.2 years; TI, 6.9 years).
- Drug treatment for anaemia in the non-TI cohort was 38.9 events per 100 patient-months and was 16.7 events per 100 patient-months in the TI cohort **(Supplementary Table 1; Please find supplementary data on treatment utilisation in the ISPOR EU app).**

HCRU and costs

- During follow-up, patients in the non-TI cohort had ~4 times more hospitalisations (mean 128.2 vs 31.9 events per 100 person-months) and more ICU admissions (mean 7.5 vs 2.8 events per 100 person-months), ER visits (mean 11.5 vs 4.4 events per 100 person-months) and outpatient visits (mean 63.1 vs 55.1 events per 100 person-months) than patients in the TI cohort (**Figure 3**).
- The mean hospital stay for patients in the non-TI cohort was substantially longer for patients in the TI cohort (391.9 vs 148.1 days per 100 person-months).
- The mean total yearly cost per patient for medical care in the non-TI cohort was almost double the cost in the TI cohort (€96,399 vs €51,832).
- This difference was largely due to greater inpatient mean costs PPPM in the non-TI cohort (€2776 vs €982), including transfusions (€1060 vs €178), and outpatient pharmacy costs (€2041 vs €1315) compared with the TI cohort (**Figure 4**).

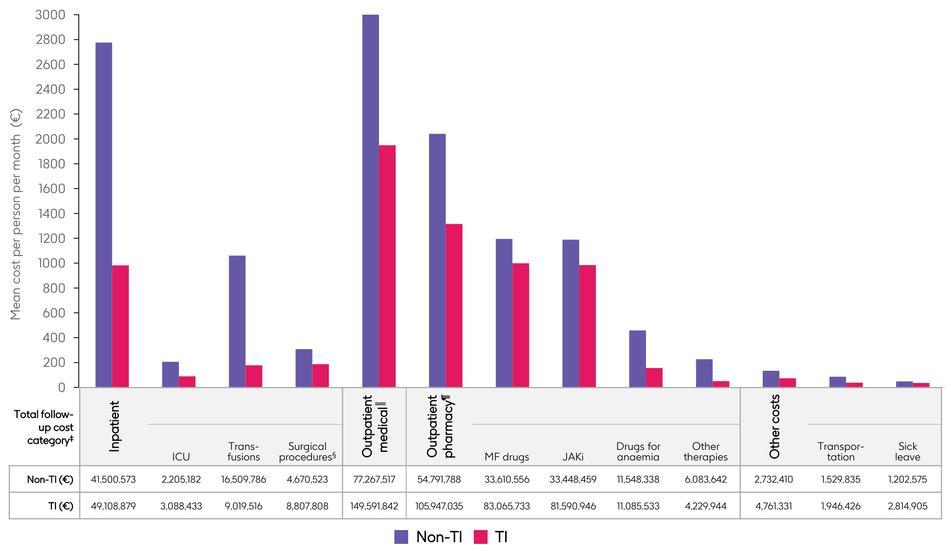
Figure 3: HCRU for non-TI and TI cohorts†



Resource utilisation category	Hospitalisations	ER visits	Outpatient visits	Laboratory services	Drugs for MF treatment	Drugs for anaemia treatment	Transfusion	Transportation	Sick leave	ICU
Non-TI, n (%)‡	944 (92.1)	613 (59.8)	905 (88.3)	965 (94.2)	779 (76.0)	533 (52.0)	763 (74.4)	730 (71.2)	79 (7.7)	487 (47.5)
TI, n (%)‡	1455 (79.4)	948 (51.8)	1739 (94.9)	1767 (96.5)	1514 (82.6)	617 (33.7)	644 (35.2)	1005 (54.9)	284 (15.5)	711 (38.8)

†Means and percentages are based on all patients, not just patients with ≥1 HCRU record. ‡n is the number of patients with ≥1 event.

Figure 4: Monthly and total healthcare costs for patients with MF in the non-TI and TI cohorts during follow-up†



Total follow-up cost category‡	Inpatient	ICU	Trans-fusions	Surgical procedures§	Outpatient medical¶	Outpatient pharmacy¶	MF drugs	JAKi	Drugs for anaemia	Other therapies	Other costs	Transpor-tation	Sick leave
Non-TI (€)	41,500,573	2,205,182	16,509,786	4,670,523	77,267,517	54,791,788	33,610,556	33,448,459	11,548,338	6,083,642	2,732,410	1,529,835	1,202,575
TI (€)	49,108,879	3,088,433	9,019,516	8,807,808	149,591,842	105,947,035	83,065,733	81,590,946	11,085,533	4,229,944	4,761,331	1,946,426	2,814,905

†Means are based on all patients and not just patients with ≥1 HCRU record. ‡Total costs are sum of all expenses for all patients in the cohort during the follow-up period. §Includes allogenic stem cell transplant. ‖Includes outpatient visits and procedures, external consultations at the hospital, ER visits without hospitalisations and outpatient laboratory services. ¶Drug cost from the outpatient pharmacy does not include ATU costs or the cost of retroceded drugs or expensive drugs not on the liste-en-sus (UCD drug cost).

Conclusions

Patients with MF requiring RBCT within a year of diagnosis have worse clinical outcomes, higher HCRU and greater healthcare costs.

Potential future treatments for MF that delay or prevent the need for patients to have RBCT may improve patient outcomes and reduce the burden of providing MF care.

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Conflicts of interest/disclosures

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Abbreviations

AML, acute myeloid leukaemia; ATU, *Autorisation Temporaire d'Utilisation* (Early Access Program); CCI, Charlson Comorbidity Index; ER, emergency room; ESA, erythropoiesis-stimulating agent; HCRU, healthcare resource utilisation; ICD-10, International Classification of Diseases, Tenth Revision; ICU, intensive care unit; IQR, interquartile range; JAKi, Janus kinase inhibitor; MF, myelofibrosis; PPPM, per patient per month; RBCT, red blood cell transfusion; SD, standard deviation; SNDS, 'Système National des Données de Santé'; TI, transfusion-independent; UCD, common dispensing units.

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