Real-World Treatment Patterns and Outcomes in Patients With Myelofibrosis Treated With First-Line Pacritinib or Ruxolitinib

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CONCLUSIONS

- **These real-world data show that first-line pacritinib (PAC) is used in patients with advanced cytopenic myelofibrosis (MF) with lower platelet counts, whereas ruxolitinib (RUX) is used in patients with higher platelet counts**
- **Despite these differences, the poor prognosis observed among patients with cytopenias, and the higher, full therapeutic dose of RUX used, PAC- and RUX-treated patients appeared to have similar 6-month survival probabilities; both treatments achieved clinically meaningful improvements in spleen size and symptom burden**

INTRODUCTION

- In patients with MF, cytopenias are associated with poor prognosis, including an inadequate response to treatment and short survival that must be considered when evaluating MF treatment options[1]
- RUX, a Janus kinase (JAK) inhibitor, is often used in the first-line setting to treat splenomegaly and symptoms but has limited benefits in patients with cytopenia[2]
- PAC, a JAK1-sparing JAK2/IRAK1/ACVR1 inhibitor, is often used as first-line therapy as it has shown consistent spleen volume reduction (SVR) and symptom improvement in clinical trials regardless of baseline platelet count,[3,4] while demonstrating anemia benefits[5] and platelet improvement in some patients[6]
- There is limited real-world evidence on patient characteristics and treatment patterns and outcomes in patients with MF receiving first-line PAC or RUX

AIM

- To describe real-world treatment patterns and outcomes in patients with MF treated with first-line PAC or RUX in the United States

METHODS

- This multisite, retrospective chart review study included patients with intermediate- or high-risk primary or secondary MF who initiated treatment with first-line PAC or RUX for ≥1 month between June 1, 2022, and July 31, 2024, with follow-up through January 31, 2025 **(Table 1)**
- Participating providers identified consecutive eligible patients at the start of the observation period. Each provider could contribute up to 10 patients across the PAC and RUX cohorts

Table 1. Key eligibility criteria

Inclusion criteria	Exclusion criteria
Received PAC or RUX as first-line JAK inhibitor for ≥1 month from June 1, 2022, through July 31, 2024	History of other malignancies (excluding non-melanoma skin cancer)
Aged ≥18 years at treatment initiation (index)	Received PAC or RUX as part of a clinical trial or for accelerated or blast phase MF
≥6-month follow-up from index*	

*Patients were not required to remain on PAC during follow-up; JAK, Janus kinase; MF, myelofibrosis; PAC, pacritinib; RUX, ruxolitinib.

- Patient characteristics, treatment patterns, palpable spleen length based on ultrasound, MF-related symptoms, hematologic outcomes (platelet counts and hemoglobin levels), and overall survival (OS) from index through Day 180 were evaluated
- Spleen length below costal margin was derived from ultrasound-based total craniocaudal spleen length minus 10 cm
- Spleen size category was based on palpation or spleen length below costal margin (not palpable/minimally palpable: <5 cm below costal margin; mild: 5-10 cm palpable; moderate: 11-20 cm palpable; severe: >20 cm palpable)
- Platelet response was defined using modified International Working Group (IWG) criteria (baseline platelets <20 x 10^9/L: increase to >20 x 10^9/L and by at least 100%; baseline platelets 20-100 x 10^9/L: absolute increase of ≥30 x 10^9/L)[7]
- Continuous variables were summarized using medians, and interquartile range (IQR) and categoric variables reported as counts and percentages. OS and survival probabilities were estimated by Kaplan-Meier survival analysis

RESULTS

Patient characteristics and treatment patterns

- Overall, 104 patients were treated with first-line PAC and 103 with first-line RUX
- Patients treated with RUX were less cytopenic and fewer patients had high bone marrow fibrosis grading and peripheral blast category (less advanced disease) at index than those treated with PAC **(Table 2)**
- However, baseline spleen size and proportion of patients with MF symptoms were comparable between the two groups **(Table 2)**

Table 2. Patient characteristics at index

	PAC (n=104)	RUX (n=103)
Age at MF diagnosis, median (IQR), years	72 (65.5, 77.0)	70 (65.0, 75.0)
Male, n (%)	57 (54.8)	53 (51.5)
Race (White), n (%)	70 (67.3)	76 (73.8)
Bone marrow fibrosis grade 3, n (%)	21/92 (22.8)	16/97 (16.5)
Platelet count (x 10^9/L), median (IQR)	45 (40, 49)	144 (111, 202)
Hemoglobin, median (IQR), g/dL	9.0 (8.2, 10.1)*	9.8 (8.9, 11.0)
Peripheral blast category (evaluable), n (%)	n=66	n=51
>0 to <2%	30 (45.5)	33 (64.7)
≥2 to <5%	34 (51.5)	18 (35.3)
>5%	2 (3.0)	0 (0)
Spleen length based on ultrasound	n=35	n=36
Median (IQR), cm	7 (6, 9)	9 (7, 10)
Baseline spleen category (evaluable), n (%)	n=63	n=62
Not palpable	4 (6.4)	4 (6.5)
Mild enlargement (5 to 10 cm palpable)	30 (47.6)	28 (45.2)
Moderate enlargement (11 to 20 cm palpable)	25 (39.7)	29 (46.8)
Severe enlargement (>20 cm palpable)	4 (6.4)	1 (1.6)
Presence of any MF symptom, %	96.2	99

*n=99; IQR, interquartile range; MF, myelofibrosis; PAC, pacritinib; RUX, ruxolitinib.

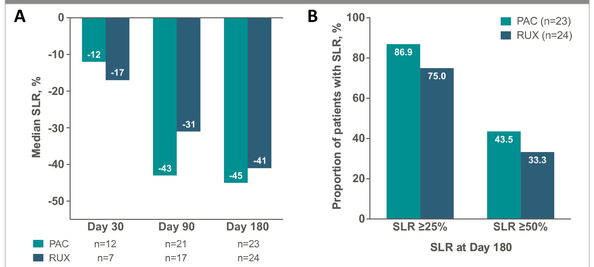
Treatment patterns

- Most patients initiated first-line PAC or RUX at the full therapeutic dose
  - 100% received 400 mg of PAC daily; 91% received ≥30 mg of RUX daily
- More patients had a ≥1 dose reduction with RUX (20/103 [19.4%]) than with PAC (15/104 [14.4%])
- After follow-up, 76/104 (73.1%) patients remained on PAC (median follow-up, 10.3 months) and 91/103 (88.4%) remained on RUX (median follow-up, 11 months)

Spleen size reduction by length and category

- Among evaluable patients with spleen length data based on ultrasound at index and Day 180, median percent reduction in spleen length below the costal margin was 45% with PAC (n=23) and 41% with RUX (n=24) **(Figure 1A)**; 43.5% (10/23) of patients who received PAC and 33.3% (8/24) who received RUX had ≥50% spleen length reduction by Day 180 **(Figure 1B)**

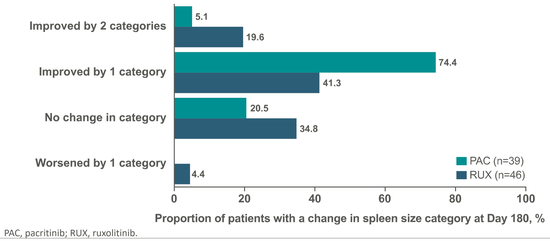
Figure 1. (A) Median spleen length reduction* (SLR) over time and (B) patients achieving ≥25% and ≥50% reduction at Day 180



*Spleen length below costal margin derived from craniocaudal spleen length minus 10 cm in patients with ultrasound spleen assessment at index and Day 180; PAC, pacritinib; RUX, ruxolitinib.

- Among patients with at least mild splenomegaly at index and spleen data at Day 180, 79.5% (31/39) treated with PAC and 60.9% (28/46) treated with RUX showed improvement in ≥1 category by any assessment **(Figure 2)**

Figure 2. Change in spleen size category from index to Day 180

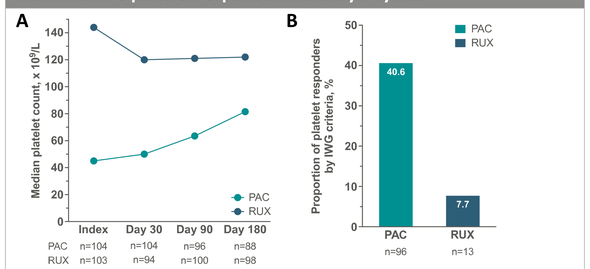


PAC, pacritinib; RUX, ruxolitinib.

Platelet response in patients with data at index and Day 180

- Median platelet (PLT) counts increased with PAC and decreased with RUX from index to Day 180 **(Figure 3A)**
- Among patients with PLT counts at index and Day 180, the median PLT count increased from index by 21 x 10^9/L with PAC (n=88) and decreased by 23.5 x 10^9/L with RUX (n=98)
- Among patients with PLT count <100 x 10^9/L at index, 40.6% (39/96) of patients treated with PAC and 7.7% (1/13) treated with RUX achieved a modified IWG PLT response criteria by Day 180 **(Figure 3B)**

Figure 3. (A) Platelet count over time and (B) patients achieving modified IWG platelet response criteria by Day 180

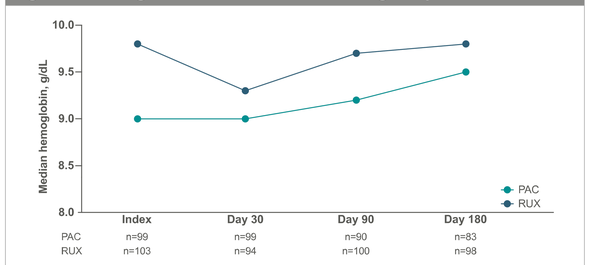


IWG, International Working Group; PAC, pacritinib; RUX, ruxolitinib.

Hemoglobin response in patients with data at index and Day 180

- By Day 180, median hemoglobin increased with PAC, whereas it stabilized with RUX after an initial decrease at Day 30 **(Figure 4)**

Figure 4. Hemoglobin levels from index through Day 180

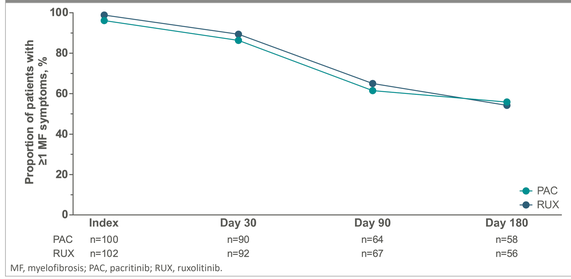


PAC, pacritinib; RUX, ruxolitinib.

Symptom reduction

- At index, more than 95% of patients in both groups had MF-related symptoms
- The number of patients reporting any MF-related symptom reduced through Day 180 in both groups **(Figure 5)**
- Most patients in both groups (PAC 80.8% [84/104]; RUX 81.6% [84/103]) also experienced a decrease in the number of MF symptoms from index to Day 180
- The median (IQR) reduction in total MF-related symptoms from index to Day 180 was 67% (50-100) for PAC (n=100) and 80% (50-100) for RUX (n=102)

Figure 5. MF-related symptoms reduction over time

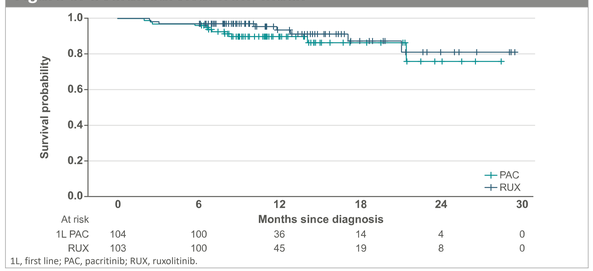


MF, myelofibrosis; PAC, pacritinib; RUX, ruxolitinib.

Overall survival

- The median (IQR) OS was similar across both groups at 10.0 (7.0-14.0) months for PAC and 11.0 (8.0-16.3) months for RUX
- The 6-month survival probability was 96.2% (95% CI, 90.1-98.5%) for PAC and 97.1% (95% CI, 91.2-99.1) for RUX **(Figure 6)**

Figure 6. Overall survival from index



1L, first line; PAC, pacritinib; RUX, ruxolitinib.

Study limitations

- As with other retrospective chart review studies, there is a risk of missing or incomplete information, as data may not be uniformly available across all treatment centers
- PLT count, hemoglobin, and spleen size evaluations were collected as part of routine medical care
- Given the limited sample size of the study, results may not be generalizable beyond the study patients

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