Consistency of Response by Baseline Platelet Count in Pacritinib-Treated Patients in the Real World: MY-PAC Analysis

PS2010

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CONCLUSIONS

- **This real-world study shows that patients with myelofibrosis (MF) treated with pacritinib experienced reductions or stabilization in spleen size, improved hematologic parameters, and reduced symptom burden, regardless of platelet counts at treatment initiation**
- **These results highlight pacritinib's potential effectiveness in patients with and without severe thrombocytopenia**

INTRODUCTION

- Pacritinib, a JAK1-sparing JAK2/IRAK1/ACVR1 inhibitor, received accelerated approval to address unmet need for patients with MF and severe thrombocytopenia (PLT count <50 x 10^9/L), and has demonstrated significant spleen volume reduction (SVR) and improved symptoms
- Data from Phase 3 trials suggest pacritinib is efficacious for spleen volume and symptom burden reduction in patients with MF, regardless of baseline platelet count[1-3]
- While pacritinib has demonstrated consistent response across a broad range of PLT counts in prior trials,[4] real-world evidence on treatment patterns and outcomes across PLT levels remains limited

AIM

- To describe real-world treatment patterns and outcomes in patients with MF treated with pacritinib stratified by PLT counts <50 and ≥50 x 10^9/L at treatment initiation.

METHODS

- This multicenter, retrospective chart review study included patients with intermediate- or high-risk primary or secondary MF initiating treatment with pacritinib between June 1, 2022, and July 31, 2024, with follow-up through January 31, 2025 **(Table 1)**.

Table 1. Key eligibility criteria

Inclusion	Exclusion
• Received pacritinib for ≥1 month from June 1, 2022 – July 31, 2024 • ≥6-month follow-up from pacritinib initiation • Aged ≥18 years at index • Treatment with pacritinib as first- or second-line JAK-inhibitor	• History of other malignancies (excluding non-melanoma skin cancer) • Received pacritinib as part of a clinical trial • Received pacritinib for accelerated or blast phase myelofibrosis

- Patients with PLT counts <50 and ≥50 x 10^9/L at pacritinib initiation (index) were included in this analysis and followed from index until the earliest of date of last contact, death, or end of study (January 31, 2025)
- Patient characteristics, treatment patterns, change in spleen size category (by palpation or ultrasound) and spleen length (ultrasound), hematologic outcomes (platelet count and hemoglobin), MF-related symptoms, and overall survival from index to post-index Day 180 were reported
- 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)
- PLT response was defined using a modified International Working Group (IWG) criteria[5]: baseline PLT count <20 x 10 ^{9}/L: increase to >20 x 10^9/L and by at least 100%; baseline PLT count 20-100 x 10 ^{9}/L: absolute increase of ≥30 x 10^9/L
- Hemoglobin response was defined as ≥1.0 g/dL or ≥1.5 g/dL increase from index through follow-up among patients with baseline hemoglobin <10 g/dL
- Continuous variables were summarized using medians, and interquartile range (IQR), and categorical variables were reported as counts and percentages. Kaplan Meier survival probabilities and 95% confidence intervals (CI) were estimated

RESULTS

Abstracting physician characteristic

- Abstraction conducted by physicians (n=41; mostly community practice) from Cardinal Health's Oncology Provider Extended Network (OPEN)

Patient characteristics and treatment patterns

- Of 168 abstracted patient charts with PLT counts at index, 73% (123/168) had PLT counts <50 x 10^9/L and 27% (45/168) with PLT counts ≥50 x 10^9/L. Most pts in both groups were treated with pacritinib as first-line JAK inhibitor **(Table 1)**
- Most patients initiated pacritinib at a daily dose of 400 mg (PLT <50 x10^9/L: 95%; 117/123, and PLT ≥50 x 10^9/L: 98%; 44/45)
- The median duration of follow-up from index was 9.8 and 7.6 months with 71% and 82% of patients remaining on pacritinib at the end of the follow-up period among those with PLT counts <50 and ≥50 x 10^9/L, respectively

Table 2. Pre-index patient characteristics

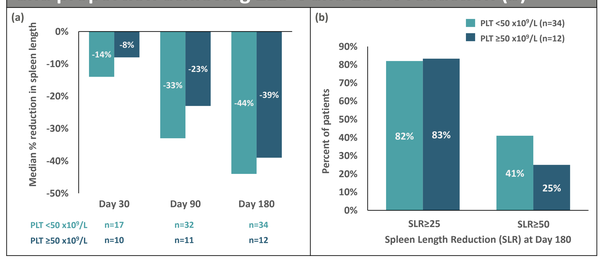
	Patients with PLT <50 x 10^9/L (N=123)	Patients with PLT ≥50 x 10^9/L (N=45)
Age (years), median (IQR)	73 (68, 78)	69 (65, 75)
Male, n (%)	74 (60.2)	24 (53.3)
Race (White), n (%)	88 (71.5)	33 (73.3)
Primary MF diagnosis, n (%)	107 (87.0)	40 (88.9)
Median time from MF diagnosis to pacritinib initiation, months (IQR)	1.8 (0.76, 13.3)	6.0 (1.3-14.3)
Pacritinib as first-line JAK inhibitor, n (%) Pacritinib as second-line JAK inhibitor, n (%) Prior Ruxolitinib Prior Momelotinib Prior Fedratinib	81 (65.9) 42 (34.1) 40 (95.2) 0 2 (4.8)	23 (51.1) 22 (48.9) 20 (90.9) 1 (4.5) 1 (4.5)
Palpable spleen length, cm (IQR)	n=48 8 (6, 12.5)*	n=21 8 (5, 11)*
Platelet count, x10^9/L, median (IQR)	43 (39, 45)	72 (53, 110)
Platelet count <100 x 10^9/L, n (%)	123 (100%)	30 (66.7%)
Hemoglobin, g/dL, median (IQR)	n=118 9.0 (8.2, 9.8)	n=45 8.8 (8.0, 9.7)
Hemoglobin <10 g/dL , n (%)	91 (77.1)	35 (77.8)
DIPPS Intermediate-1 risk (1-2 points), n (%) Intermediate-2 risk (3-4 points), n (%) High-risk (5-6 points), n (%)	n=86 18 (20.9) 48 (55.8) 20 (23.3)	n=28 7 (25) 11 (39.3) 10 (35.7)

*Patients with ultrasound-derived palpable spleen length within 14 days of index; DIPPS, Dynamic International Prognostic Scoring System; IQR, interquartile range; MF, myelofibrosis.

Spleen size reduction by length and by category

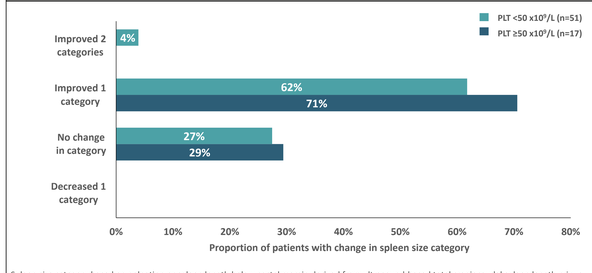
- Among patients with spleen length based on ultrasound at index and Day 180, the median percent reduction of spleen length below the costal margin was 44% (n=34) and 39% (n=12) **(Figure 1a)** and 41% (14/34) and 25% (3/12) of patients achieved ≥50% spleen length reduction by Day 180 **(Figure 1b)** for those with PLT counts <50 and ≥50 x 10^9/L respectively
- A majority of patients with at least mild splenomegaly at index and spleen data at Day 180 achieved 1 category reduction in spleen size **(Figure 2)**:
  - 62% (31/51) of patients with PLT <50 x 10^9/L
  - 71% (12/17) of patients with PLT ≥50 x 10^9/L

Figure 1. Spleen length reduction* (SLR): median change (a) and proportion achieving ≥25% and ≥50% reduction (b)



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

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



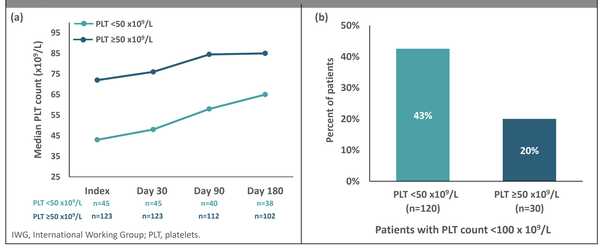
Spleen size category based on palpation or spleen length below costal margin derived from ultrasound-based total craniocaudal spleen length minus 10 cm (not palpable/minimally palpable <5 cm below costal margin; mild: 5-10 cm palpable; moderate: 11-20 cm palpable; severe: >20 cm palpable). Note: all patients with at least mild splenomegaly at index and day 180 data were analyzed.

PLT, platelets.

PLT response with pacritinib

- Median PLT count was stable from index to Day 180 in both groups **(Figure 3a)**
- Among patients with PLT counts at index and Day 180, the median change in PLT count from index was 24 x 10^9/L among patients with PLT <50 x 10^9/L at index (n=102) and 10 x 10^9/L among patients with PLT ≥50 x 10^9/L (n=38)
- Among patients with PLT count <100 x 10^9/L at index, 43% (51/120) with PLT counts <50 x 10^9/L and 20% (6/30) with PLT counts ≥50 x 10^9/L achieved a modified IWG PLT by Day 180 **(Figure 3b)**

Figure 3. PLT count over time (a) and modified IWG criteria by Day 180 (b)

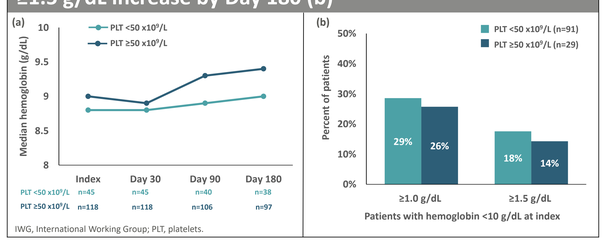


IWG, International Working Group; PLT, platelets.

Hemoglobin response with pacritinib

- Hemoglobin remained stable over time, with modest increase by Day 180 in both groups **(Figure 4a)**
- Among patients with hemoglobin <10 g/dL at index:
  - Hemoglobin increased by a median 0.6 g/dL and 0.8 g/dL in patients with PLT count <50 and ≥50 x 10^9/L respectively by Day 180
  - Roughly one-third of patients achieved ≥1.0 g/dL increase in hemoglobin in both groups, and >50% of these patients achieved ≥1.5 g/dL increase by Day 180 **(Figure 4b)**

Figure 4. Hemoglobin levels over time (a) and ≥1.0 g/dL and ≥1.5 g/dL increase by Day 180 (b)

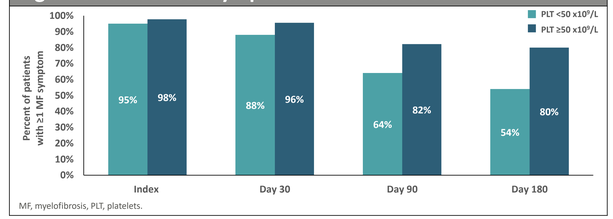


IWG, International Working Group; PLT, platelets.

Symptom Data

- At index, most patients in both groups had ≥1 MF-related symptom (PLT <50 x 10^9/L: 95%; and PLT ≥50 x 10^9/L: 98%)
- A reduction in the number of patients reporting any MF-related symptom was observed through Day 180 in both groups **(Figure 5)**
- The median reduction in total MF-related symptom count was 67% (IQR: 50-100) for patients with PLT <50 x 10^9/L and 67% (IQR: 33-75) for patients with PLT ≥50 x 10^9/L

Figure 5. MF-related symptoms reduction over time

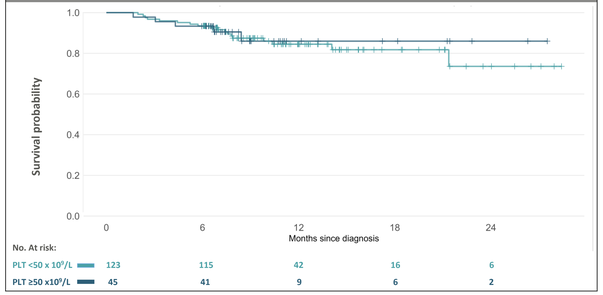


MF, myelofibrosis, PLT, platelets.

Overall survival

- By the end of the follow-up period, 85% (105/123) of patients with PLT <50 x 10^9/L were alive with a 6-month survival probability of 93.5% (95% CI: 87.4-96.7) **(Figure 6)**
- A similar proportion of patients with PLT counts ≥50 x 10^9/L were alive at the end of follow-up (89%; 40/45) with a comparable 6-month survival probability of 93% (95% CI: 81-98)

Figure 6. Overall survival from index



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

REFERENCES: **1.** Mascarenhas J, et al. *JAMA Oncol*. 2018;4(5):652-9. **2.** Mesa RA, et al. *Lancet Haematol*. 2017;4(5):e225-e36. **3.** Gerds AT, et al. *Blood Adv*. 2020;4(22):5825-35. **4.** Gagelmann N, et al. *Clin Lymphoma Myeloma Leuk*. 2024;24(11):796-803. **5.** Platzbecker et al. *Blood*. 2019;133(10):1020-1030,

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