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Real-World Use of Pacritinib in Myelofibrosis: Results from the TriNetX Analysis on Patient Characteristics, Treatment Patterns, and Outcomes in Patients with Platelets ≥50×10⁹/L at Initiation (TRIPAC)

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CONCLUSIONS

- **In this real-world retrospective analysis of patients with myelofibrosis (MF) and platelet (PLT) counts ≥50×10⁹/L at index, treatment with pacritinib (PAC) resulted in an improvement of hematologic parameters in a notable proportion of patients at 6 months.**
- **These findings provide real-world evidence supporting PAC use in patients with MF and PLT ≥50x10⁹/L at initiation.**

BACKGROUND

- Pacritinib, a JAK1-sparing inhibitor of JAK2, IRAK1, and ACVR1, received accelerated approval for use in patients with MF who have severe thrombocytopenia (PLT <50×10⁹/L), where it has demonstrated spleen volume reduction and symptom improvement[1,2]
- In Phase 3 clinical trials, PAC reduced splenomegaly and disease symptoms in patients with MF across a broad range of baseline PLT counts, including patients with PLT ≥50×10⁹/L[1,3]
- Cytopenias in MF limit the tolerability, dose intensity, and durability of other JAK inhibitors, leaving a high unmet need across the cytopenic MF spectrum, including patients with PLT ≥50×10⁹/L
- Real-world evidence describing PAC treatment patterns and hematologic and survival outcomes in patients with PLT ≥50×10⁹/L at initiation remains limited

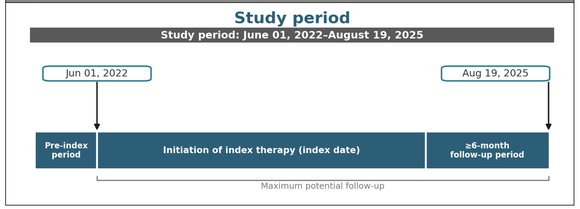
AIM

- To describe real-world patient characteristics, treatment patterns, hematologic outcomes (PLT and hemoglobin [Hb]), and overall survival (OS) in patients with MF and PLT ≥50×10⁹/L at index who initiated PAC

METHODS

- Retrospective analysis of de-identified electronic health record (EHR) data from the TriNetX US Dataworks network
- Included adults with an MF diagnosis and PLT ≥50×10⁹/L at PAC initiation (index) on or after June 1, 2022
- Patients were required to have ≥2 PAC prescriptions and ≥6 months of follow-up unless death occurred earlier, but were not required to remain on PAC for the full 6 months (**Figure 1)**

Figure 1. Study design



- Within this population, a subgroup with concurrent anemia (PLT ≥50×10⁹/L and Hb <10 g/dL at index) was also examined
- Index PLT and Hb were defined as the most recent laboratory value prior to the index date, within 30 days prior to or on the index date
- Outcomes included changes in PLT and Hb, and OS, from index; for Day 90 and Day 180 assessments, labs from the corresponding post-index window were used, selecting the value closest to, but prior to, the timepoint when multiple were available; outcomes were observed among patients who continued to be prescribed PAC
- Hematologic change was categorized as improved, stable, or worsened by comparing the category at index with the category at the specified timepoint (using the laboratory value closest to that timepoint), using the following strata — Hb (g/dL): <6.5, 6.5 – <8.0, 8.0 – <10.0, ≥10; PLT (×10⁹/L): <50, 50 – <100, 100 – <150, ≥150
- Categorical Hb response was assessed in patients with concurrent anemia (Hb <10 g/dL), and categorical platelet response in patients with index PLT 50–149×10⁹/L; patients with Hb ≥10 g/dL or PLT ≥150×10⁹/L at index were excluded from the respective categorical analyses
- OS was estimated by the Kaplan-Meier method from the second PAC prescription, required of all patients, to avoid immortal time bias

RESULTS

Patient characteristics and treatment patterns

- Patients had received a median of 2 (IQR: 1–2) prior lines of MF-directed therapy
- Of 69 patients with PLT ≥50×10⁹/L, PAC was used first-line in 30.4% (21/69) and second-line in 52.2% (36/69), third-line in 13.0% (9/69), and fourth-line in 4.3% (3/69)
- Among the 48 patients who received PAC second-line or later, 95.8% (46/48) had received ruxolitinib, 16.7% (8/48) fedratinib, and 8.3% (4/48) momelotinib (categories not mutually exclusive)
- At index, median PLT was 123×10⁹/L (IQR: 71, 201) and Hb was 9.3 g/dL (IQR: 7.9, 10.7); 62.9% (39/62) had Hb <10 g/dL

Table 1. Patient characteristics, treatment patterns, and hematologic values at index

Characteristic	PLT ≥50×10⁹/L (N=69)	PLT ≥50×10⁹/L & Hb <10 g/dL (N=39)
Age at index, years — median (Q1– Q3)	72 (68–77)	72 (66–78)
Male, n (%)	34 (49.3)	20 (51.3)
Follow-up, months — median (Q1–Q3)	15.4 (10.1–23.7)	14.8 (9.5–20.3)
Primary MF, n (%)	33 (47.8)	18 (46.2)
Prior lines of MF therapy — median (Q1–Q3)	2 (1–2)	2 (1–2)
PAC as 1st JAK inhibitor, n (%)	21 (30.4)	9 (23.1)
Prior ruxolitinib (of those with prior JAKi), n (%)	46/48 (95.8)	29/30 (96.7)
PLT, ×10⁹/L — median (Q1–Q3)	123 (71–201)	123 (77–226)
PLT <150×10⁹/L, n (%)	43 (62.3)	26 (66.7)
Hb, g/dL — median (Q1–Q3)	9.3 (7.9–10.7)	8.1 (7.4–9.3)
Hb <10 g/dL, n (%)	39 (62.9)	39 (100)
Hb <8 g/dL, n(%)	16 (25.8)	16 (41.0)
Hb <6.5 g/dL, n(%)	2 (3.2)	2 (5.1)

Hematologic changes at Day 90 and Day 180

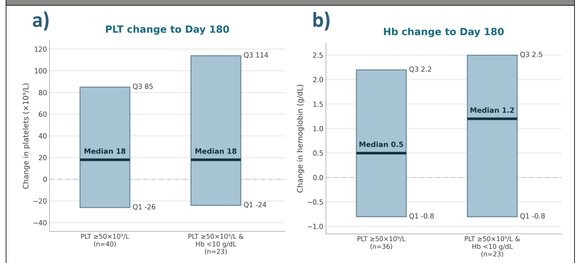
At Day 90:

- Among patients with values at index and Day 90, median PLT change was 0×10⁹/L (IQR: −37, +60; n=42), with 25% achieving increases ≥60×10⁹/L
- Median Hb change was +0.4 g/dL (IQR: −0.7, +1.1; n=38), with 25% achieving increases ≥1.1 g/dL
- Among patients with PLT ≥50×10⁹/L and Hb <10 g/dL at index, median Hb increased by 0.3 g/dL at Day 90 (IQR: −0.7, +1.3; n=24), with 25% achieving increases ≥1.3 g/dL

At Day 180 (Figure 2a and 2b):

- Among patients with values at index and Day 180, median PLT change was +18×10⁹/L (IQR: −26.0, +85.0; n=40), with 25% achieving increases ≥85×10⁹/L
- Median Hb change was +0.5 g/dL (IQR: −0.8, +2.2; n=36), with 25% achieving increases ≥2.2 g/dL
- Among patients with PLT≥50×10⁹/L and Hb<10 g/dL at index, median Hb increased by 1.2 g/dL at Day 180 (IQR: −0.8, +2.5; n=23), with 25% achieving increases ≥2.5 g/dL

Figure 2. Change from index to Day 180 in platelets (left) and hemoglobin (right)

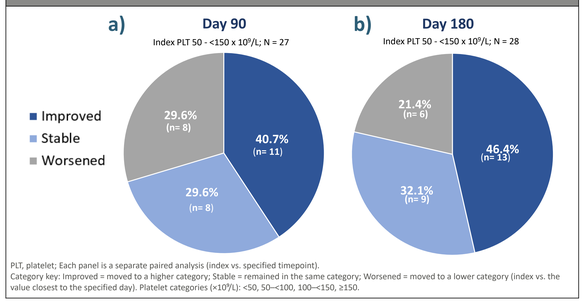


Hb, hemoglobin; PLT, platelet.
Shown as the interquartile range (Q1–Q3, box) with the median (bold line); the dashed line marks no change from index. Paired values only; denominators (n) shown per group.

Platelet response category in patients with thrombocytopenia

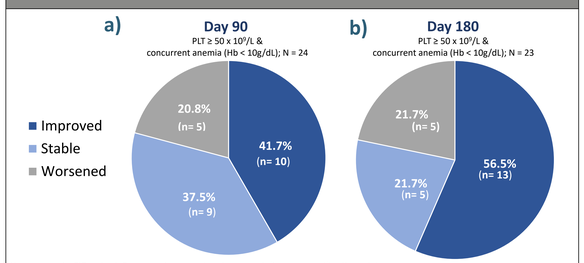
- At Day 90, 70% (19/27) of patients with thrombocytopenia (index PLT 50–149×10⁹/L) had improved or stable platelet category (40.7% [11/27] improved, 29.6% [8/27] stable) **(Figure 3a)**
- At Day 180, 79% (22/28) had improved or stable platelet category (46.4% [13/28] improved, 32.1% [9/28] stable) **(Figure 3b)**
- Among patients with PLT ≥150×10⁹/L at index, 86.7% (13/15) remained ≥150×10⁹/L at Day 90 and 58.3% (7/12) at Day 180 (91.7% [11/12] remained ≥100×10⁹/L at Day 180)

Figure 3. PLT response category at (a) Day 90 and (b) Day 180 in patients with thrombocytopenia



PLT, platelet; Each panel is a separate paired analysis (index vs. specified timepoint).
Category key: Improved = moved to a higher category; Stable = remained in the same category; Worsened = moved to a lower category (index vs. the value closest to the specified day). Platelet categories (×10⁹/L): <50, 50–<100, 100–<150, ≥150.

Figure 4. Hb response category at (a) Day 90 and (b) Day 180



Hb, hemoglobin; PLT, platelet; Each panel is a separate paired analysis (index vs. specified timepoint).
Category key: Improved = moved to a higher category; Stable = remained in the same category; Worsened = moved to a lower category (index vs. the value closest to the specified day). Hemoglobin categories (g/dL): <6.5, 6.5–<8.0, 8.0–<10.0, ≥10.

Hemoglobin response category in patients with concurrent anemia

- At Day 90, 79% (19/24) of patients with concurrent anemia (Hb <10 g/dL) had improved or stable Hb category (41.7% [10/24] improved, 37.5% [9/24] stable) **(Figure 4a)**
- At Day 180, a similar proportion (78.2%, 18/23) had improved or stable Hb category (56.5% [13/23] improved, 21.7% [5/23] stable) **(Figure 4b)**
- Among patients with Hb ≥10 g/dL at index, most remained ≥10 g/dL — 78.6% (11/14) at Day 90 and 76.9% (10/13) at Day 180

Overall Survival

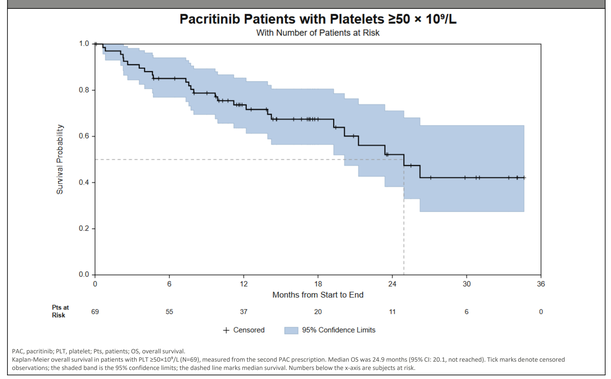
Table 2. Overall survival (from second PAC prescription)

Overall survival	PLT ≥50×10⁹/L (N=69)
KM median OS, months (95% CI)	24.9 (20.1, NR)
Censored, n (%)	43 (62.3)
Events (deaths), n (%)	26 (37.7)

KM, Kaplan-Meier; NR, not reached; PAC, pacritinib; OS, overall survival. OS measured from the second PAC prescription; patients alive at end of follow-up censored at last encounter.

- Median OS was 24.9 months (95% CI: 20.1, not reached) in patients with PLT ≥50×10⁹/L over a median follow-up of 15.4 months, with 62.3% (43/69) of patients remaining alive at last observation **(Table 2 & Figure 5)**

Figure 5. Overall survival (Kaplan-Meier)



PAC, pacritinib; PLT, platelet; Pts, patients; OS, overall survival.
Kaplan-Meier overall survival in patients with PLT ≥50×10⁹/L (N=69), measured from the second PAC prescription. Median OS was 24.9 months (95% CI: 20.1, not reached). Tick marks denote censored observations; the shaded band is the 95% confidence limits; the dashed line marks median survival. Numbers below the x-axis are subjects at risk.

Study limitations

- Limited sample size may reduce generalizability beyond the study population
- PLT and Hb evaluations were collected as part of routine medical care and not at standardized time intervals
- Spleen size, symptom burden, RBC transfusion status, and molecular/risk features were not available to fully characterize clinical characteristics and treatment outcomes
- PAC dose was not captured; however, a recent real-world study (MY-PAC) reported that >95% of pacritinib-treated patients received the full 400 mg total daily dose, largely in community practices[4]

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