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Real-World Treatment Patterns and Clinical Outcomes in Patients with Myelofibrosis Treated with Pacritinib (PAC) with platelets ≥50x10^9/L at PAC initiation: Interim results from the MY-PAC Study

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CONCLUSIONS

- In this interim real-world analysis, PAC demonstrated clinical benefit in MF patients with PLT ≥50 x10^9/L, including reductions in spleen size category, stabilization or improvement in hematologic parameters.
- These findings support the utility of PAC in patients with moderate thrombocytopenia.

BACKGROUND

- Pacritinib (PAC), a JAK1 sparing JAK2/IRAK1/ACVR1 inhibitor, received accelerated approval to address an unmet need for patients with MF and severe thrombocytopenia (PLT counts <50 x10^9/L), and has demonstrated significant spleen volume reduction (SVR) and improved symptoms.
- Data from Phase 3 trials suggest PAC is efficacious for SVR and symptom burden reduction in patients with MF, regardless of baseline platelet count.[1-3]
- PAC has been studied across broader PLT count ranges in prior trials which suggest consistent response, however, understanding PAC treatment patterns and outcomes in real-world patient populations with higher PLT counts is important.

AIMS

- To evaluate real-world treatment patterns and outcomes in MF patients treated with PAC who had PLT counts ≥50x10^9/L at treatment initiation.

METHODS

- This multicenter, retrospective chart review study included United States (US) patients with intermediate- or high-risk primary or secondary MF treated with PAC between June 1, 2022, and June 3, 2024.
  - Patients had to be at least 18 years of age at the time of PAC initiation (index), received treatment with PAC for at least one month, and a minimum 6 months of follow-up from PAC initiation, except for death.
  - Patients were excluded if they had a history of diagnosis of any other malignancies, excluding non-melanoma skin cancer, received PAC in a therapeutic clinical trial, or received PAC for accelerated or blast phase MF.
- Notable improvements in lab values were assessed (i.e. PLT increase based on IWG response criteria[4] and Hb increase of ≥1.0g/L relative to index).
- Patients with PLT ≥50 x10^9/L at PAC initiation (index) were included in this interim analysis and followed from index until the earliest of date of last contact, death, or study end (Dec 3, 2024).
- Patient demographic characteristics, treatment patterns, spleen size (categorized as not palpable [NP]: including minimally palpable < 5 cm below costal margin; mild: 5-10 cm palpable; moderate: 11-20 cm palpable; and severe: >20 cm palpable), hematologic outcomes (PLT and Hb), and overall survival (OS) from index through post-index Day 180 were described.
  - Index in this study is the most recent data available at index or within 14 days of index.
  - Variables were described using counts, percentages, medians, interquartile range (IQR), and Kaplan Meier survival probabilities.

RESULTS

Abstracting physician characteristics

- Physicians (n=40) from Cardinal Health's Oncology Provider Extended Network (OPEN) were representative of all US regions.
- Thirty-four physicians (85%) came from community practices.
- The median number of provider years in practice was 15.5 years (IQR: 12-20).

Patient characteristics (Table 1)

- Thirty-five patients with MF and PLT ≥50 x10^9/L were treated with PAC as the first line (1L; n=18) or second-line (2L; n=17) janus kinase (JAK)-inhibitor.
- At MF diagnosis, median age was 68 years (IQR: 62 to 74). Most patients were male (51.4%) and White (80%).

RESULTS

Patient characteristics, continued (Table 1)

- Median (IQR) time from MF diagnosis to index was 6 months (0.7 to 15.9) overall.
  - Median (IQR) time from MF diagnosis to PAC initiation was 1 month (0.5 to 2.0) for 1L initiators and 16 months (13.7 to 44.7) for 2L initiators.
- Median follow-up was 8 months, and 80% of patients were still on PAC at the end of the 180-day observation period.

Table 1: Pre-Index Patient Characteristics

	PAC treated patients (N=35)
Age at PAC initiation (years)	
Median (Q1-Q3)	70 (65.0-75.0)
Gender at birth (n, %)	
Male	18 (51.4)
Race (n, %)*	
Asian	2 (5.7)
Black or African American	4 (11.4)
Native Hawaiian or Other Pacific Islander	1 (2.9)
White	28 (80.0)
Time from initial MF diagnosis to initiation of index therapy (months)	
Median (Q1-Q3)	6 (0.7-15.9)

*Categories of response not mutually exclusive, total may sum to more than column total.

Spleen size reduction (Figure 1 & Table 2)

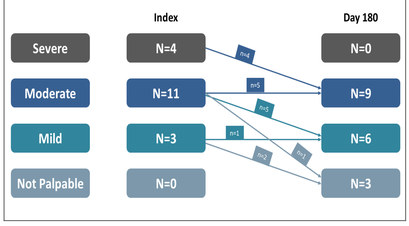
- Of 18 patients evaluated at index and Day 180 with at least mild splenomegaly at index, 12 patients (66.7%) achieved a reduction in spleen size category by Day 180.
  - Of 3 patients with mild splenomegaly, 2 patients (66.7%) became NP and 1 patient remained mild.
  - Of 11 patients with moderate splenomegaly, 6 patients (54.5%) achieved a reduction in spleen size category (NP: n=1; mild: n=5) and 5 patients remained moderate.
  - All 4 patients with severe splenomegaly at index achieved a reduction to moderate splenomegaly at Day 180.
  - No patient had worsening of spleen category through both Day 90 as well as Day 180.

Table 2: Spleen Response

	PAC treated patients (N=35)	PAC as 1st JAK inhibitor (n=18)	PAC as 2nd JAK inhibitor (n=17)
Spleen size changes from index to Day 180 post-index*			
n, % evaluated	18 (51.4)	9 (50.0)	9 (53.0)
No change in spleen size	6 (33.3)	2 (22.2)	4 (44.4)
Mild to non palpable	2 (11.1)	1 (11.1)	1 (11.1)
Moderate to non palpable	1 (5.6)	1 (11.1)	0 (0)
Moderate to mild	5 (27.8)	4 (44.4)	1 (11.1)
Severe to moderate	4 (22.2)	1 (11.1)	3 (33.3)

*Among patients with available data at index and Day 180 post-index

Figure 1: Change in Spleen Size Category from Index to Day 180 Post-Index



Hemoglobin

- Median Hb was 9.0 g/dL (IQR: 8.0 to 9.2) at index, and most patients had Hb <10 g/dL (85.7%; 30/35).
  - Increases in Hb were seen from index through Day 180 [10% median increase (IQR: -1.1% to 12.5%)] (**Figure 2**).
  - By Day 180, 28.6% (10/35) of patients achieved ≥1.0 g/dL increase in Hb relative to index (**Figure 3**).

Figure 2: Median Percent Change in Hb and PLT Count From Index to Day 180 Post-Index

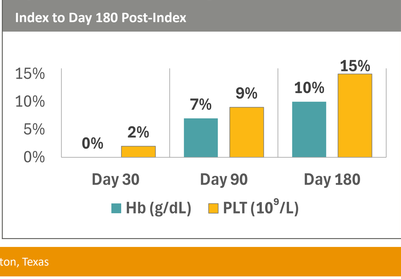
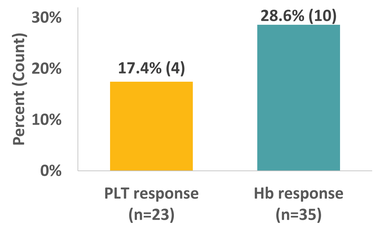


Figure 3: Percent of Patients with Notable Improvement in Lab Values from Index to Day 180 Post-Index



Platelet Count

- Median PLT count at index was 60 x10^9/L (IQR: 51.0 to 110.0).
- The majority of patients (65.7%; 23/35) presented with moderate thrombocytopenia (PLT count ≥50 to <100 x10^9/L).
- PLT count showed median (IQR) improvement of 15% (6.8% to 30%) from index through Day 180 (**Figure 2**).
  - By Day 180, 17.4% (4/23) of patients with moderate thrombocytopenia achieved an IWG PLT response with an absolute increase of ≥30 x10^9/L (**Figure 3**).

Survival

- Thirty-one patients (88.6%) were alive through the end of the study period.
- 180-day survival probability from PAC initiation was 91.4% (95% CI: 75.7-97.2) (**Figure 4** and **Table 3**).

Figure 4: Overall Survival at 6 months of Patients Treated with Pacritinib

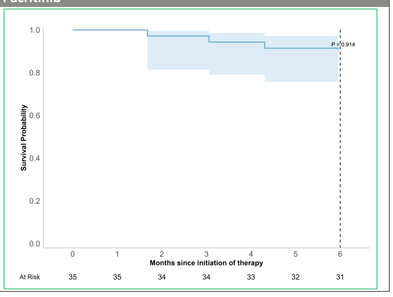


Table 3: Overall Survival

	PAC treated patients (N=35)	PAC as 1st JAK inhibitor (n=18)	PAC as 2nd JAK inhibitor (n=17)
OS from PAC initiation[1]			
N of censored	31 (88.6)	17 (94.4)	14 (82.4)
N of events	4 (11.4)	1 (5.6)	3 (17.6)
KM median, 95% CI	NR [NE-NE]	NR [8.4-NE]	NR [NE-NE]
OS at Day 180			
N at risk	31 (88.6)	18 (100)	13 (76.5)
Point estimate, 95% CI	0.9 [0.76-0.97]	1.0 [1.0-1.0]	0.8 [0.55-0.94]

[1]Time from index initiation to date of death (event). Patients still alive at the end of follow-up/study end date will be censored on the date of last encounter.

Study Limitations

- Study patients were required to have at least 6 months of post-index data, except for cases of death. Patients lost to follow-up from PAC initiation may differ from those who continued their care at the same medical center.
- PLT, Hb, and spleen size evaluations were collected as part of routine medical care and not at standardized time intervals.
- Observational nature of the study limits the ability to infer causal relationships.

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Disclosures

Puja Aggarwal, Emily Levine, Djibril Liassou, Adina Gegprifti, and Bruce Feinberg are employees of Cardinal Health. Bruce Feinberg has stock ownership in Cardinal Health. Abiola Oladapo, Michael Marrone, Purvi Suthar, and Michael Vredenburg are employees of Sobi Inc. Gerard Hoehn was an employee of Sobi Inc. at the time of study conduct.

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