Hematologic Improvement Experienced by Pacritinib-Treated Patients With Myelofibrosis in Real-World Clinical Settings

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CONCLUSIONS

- Almost half of pacritinib (PAC)-treated patients with thrombocytopenia experienced a platelet (PLT) response as defined per the International Working Group (IWG) criteria, with median PLT count increasing by almost 50% in this real-world analysis
- Patients who achieved PLT response also experienced an increase in median hemoglobin (Hb) levels by >1 g/dL
- Both platelet count and Hb levels remained stable in those who did not experience a platelet response

BACKGROUND

- Myelofibrosis (MF) is a rare myeloproliferative neoplasm characterized by a complex symptom profile (cytopenia-related fatigue, fever, weight loss, bleeding, bone pain, etc), splenomegaly, potential for leukemic progression, and shortened survival[1]
- Most patients with MF experience moderate to severe thrombocytopenia (PLT counts <100 x 10^9/L) which correlates with poor prognosis[2,3]
- PAC, a JAK1-sparing inhibitor of JAK2/IRAK1/ACVR1, is approved by the US Food and Drug Administration for the treatment of patients with MF and severe thrombocytopenia[1,4]
- In clinical trial settings, treatment with PAC is associated with PLT stability and, in some cases, improvement, but real-world evidence on hematologic response is limited[5,6]

AIM

- To evaluate treatment patterns and outcomes in patients with MF and thrombocytopenia treated with PAC experiencing a PLT response in real-world clinical practice

METHODS

- Integra-PrecisionQ database, including electronic health data and practice management data (80% community oncology practices) was used to select patients with MF (based on *International Classification of Disease, Tenth Revision* [ICD-10] diagnostic codes: D47.4, D75.81, and D47.1) treated with PAC (index) between June 1, 2022, and August 31, 2023, in real-world clinical settings
- Data were extracted after the index date to the end of data availability, end of study (October 31, 2023), or death, whichever occurred first
- The analysis was conducted on a subset of patients with a PLT count <100 x 10^9/L at index who were alive and had data available for ≥90 days post-index
  - PLT response was defined per IWG criteria at any time within 90 days of PAC initiation:
    - Baseline PLT <20 x 10^9/L: increase to >20 x 10^9/L and by at least 100%
    - Baseline PLT 20–100 x 10^9/L: an absolute increase of ≥30 x 10^9/L
  - Treatment-related outcomes assessed included:
  - PLT and Hb levels from post-index day 90 through the end of the study period
  - Overall survival (OS) probabilities and 95% CIs from post-index day 90 were estimated using Kaplan-Meier method
    - Patients were followed from post-index day 90 until the end of data availability or death
- Continuous variables were summarized using median, and interquartile range, and categorical variables were described using counts and percentages

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RESULTS

- Of 119 patients treated with PAC with available laboratory data at index and follow-up, 61 patients had PLT count <100 x 10^9/L at index and were alive for ≥90 days post-index
- Of the 61 patients, 28 (45.9%) met the criteria for PLT response by post-index day 90
- The median follow-up from MF diagnosis, and time from MF diagnosis to index was similar for PLT responders (PLT-R) and non-responders (PLT-NR) **(Table 1)**
- The median follow-up from index was longer in PLT-R group **(Table 1)**
- PLT-R had a higher median PLT count at index and a majority of patients had a PLT count of 50-100 x 10^9/L **(Table 1)**
- Median Hb levels at index were comparable among both groups **(Table 1)**

Table 1. Baseline treatment characteristics by PLT response

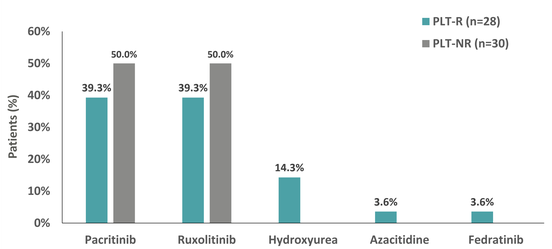
	PLT-R (n=28)	PLT-NR (n=33)
Age at PAC initiation (index), years		
Median (Q1, Q3)	80 (71, 82.5)	75 (66, 80)
Sex, n (%)		
Male	18 (64.3)	20 (60.6)
Race, n (%)		
White	18 (64.3)	25 (75.8)
Other/Unknown	10 (35.7)	8 (24.2)
Follow-up from MF diagnosis, months		
Median (Q1, Q3)	14.1 (9.2, 45.8)	14 (8.1, 54.5)
Time from MF diagnosis to PAC initiation (index), months		
Median (Q1, Q3)	7.1 (0.1, 35.7)	6.8 (0.5,47.3)
Follow-up from PAC initiation (index), months		
Median (Q1, Q3)	8.9 (4.5, 11.5)	6.0 (5.2, 10.8)
PLT count at PAC initiation (index)		
Median (Q1, Q3)	64.5 (45.0, 81.0)	48.0 (30.0, 68.0)
PLT count <50 x 10^9/L , n (%)	10 (35.7)	15 (45.5)
PLT count 50-100 x 10^9/L, n (%)	18 (63.6)	18 (54.6)
Hb level at PAC initiation (index)		
Median (Q1, Q3)	8.8 (7.4, 10.4)	9.2 (8.3, 10.9)

Hb, hemoglobin; MF, myelofibrosis; NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Treatment patterns with pacritinib

- First-line use of PAC and ruxolitinib were similar in PLT-R group **(Figure 1)**
- PLT-NR group was more likely to receive first-line ruxolitinib compared to PLT-R group (**Figure 1**)

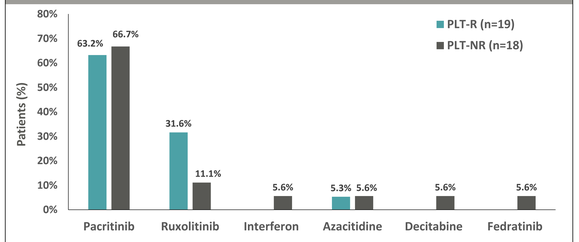
Figure 1. First-Line of treatment by PLT response



NR, non-responder; PLT, platelets; R, responder.

- PAC was the most common second-line treatment in both subgroups **(Figure 2)**

Figure 2. Second-Line of treatment by PLT response



NR, non-responder; PLT, platelets; R, responder.

- Time from MF diagnosis to first-line treatment with PAC, and the interval between prior line of treatment to second-line treatment with PAC was similar in PLT-R and PLT-NR groups **(Table 2)**
- The median duration of PAC treatment was greater in PLT-R group than in PLT-NR group with similar patterns of duration of treatment with PAC restricting to patients with ≥6 months follow-up (**Table 2**)

Table 2. Time to start and duration of PAC treatment

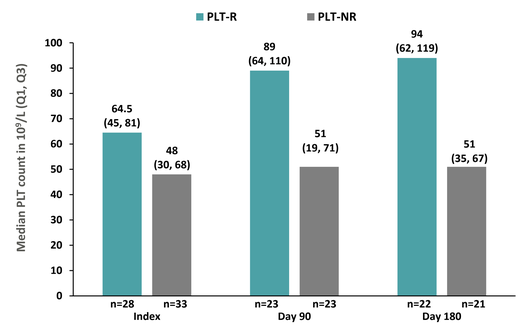
	PLT-R (n=28)	PLT-NR (n=33)
Time from MF diagnosis to 1L PAC initiation, months	**n=11**	**n=15**
Median (Q1, Q3)	0 (0, 0.5)	0.5 (0, 1.8)
Time between end of previous MF therapy and 2L PAC initiation, months	**n=12**	**n=14**
Median (Q1, Q3)	0.03 (0.03, 0.04)	0.03 (0.03, 6.7)
Duration of PAC treatment, months		
Median (Q1, Q3)	7.2 (3.6, 11.1)	5.6 (2.7, 7.5)
Duration of PAC treatment, (≥6-months follow-up), months	**n=18**	**n=18**
Median (Q1, Q3)	10.1 (7.5, 11.5)	6.9 (3.9, 11.0)

NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Platelet response with pacritinib treatment

- In the PLT-R group, the median PLT count increased from 64.5 x 10^9/L at index to 89 x 10^9/L at post-index day 90 and 94 x 10^9/L at day 180 **(Figure 3)**
- The median PLT count remained stable from index (48 x 10^9/L) through post-index days 90 (51 x 10^9/L) and 180 (51 x 10^9/L) in the PLT-NR group **(Figure 3)**

Figure 3. Impact of PAC on PLT counts by PLT response

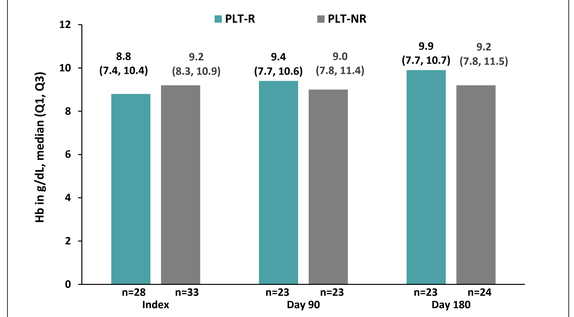


NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Hemoglobin response with pacritinib treatment

- In the PLT-R group, the median Hb increased from 8.8 g/dL at index date to 9.4 g/dL at post-index day 90 and 9.9 g/dL at post-index day 180 **(Figure 4)**
- The median Hb remained stable from index (9.2 g/dL), to post-index day 90 (9.0 g/dL), and post-index day 180 (9.2 g/dL) in the PLT-NR group (**Figure 4**)

Figure 4. Impact of PAC on Hb levels by PLT response

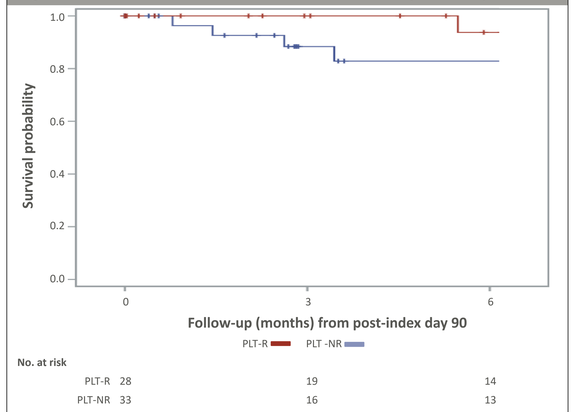


NR, non-responder; PLT, platelets; R, responder.

Overall Survival

- Overall survival was 92.9% (26/28) in the PLT-R group and 89.9% (29/33) in the PTL-NR group through the end of the observation period
- From day 90, 6-month OS was 93.7% (95% CI: 63.2, 99.0) for patients with PLT response and 82.9% (95% CI: 59.8, 93.3) for patients without PLT response **(Figure 5)**

Figure 5. PAC treatment and overall survival by PLT response



NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Limitations

- As with other retrospective database studies, there is a risk of missing or incomplete information, as data may not have been uniformly available for all the patients
- Given the limited sample size of the study, results may not be generalizable beyond the study patients

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DISCLOSURES: MM, AO, PS and MV, are Sobi employees. LM is an employee of IntegraConnect, TS, AM, JZ were employees of IntegraConnect when the work was done. RR and JM are Sobi consultants.

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