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

PF849

Michael Marrone,[1] Samantha Eiffert,[2] Lisa Morere,[2] Abiola Oladapo,[1] Purvi Suthar,[1] Teena Sura,[2] Anupama Vasudevan,[2] Michael Vredenburg,[1] Jia Zeng,[2] Raajit Rampal,[3] John O. Mascarenhas[4]

[1]Sobi Inc., Waltham, MA, USA; [2]IntegraConnect, PrecisionQ, West Palm Beach, FL, USA; [3]Memorial Sloan Kettering Cancer Center, New York, NY; [4]Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai, New York, NY, USA.

CONCLUSIONS

- **More than 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 more than 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), splenomegaly, potential for leukemic progression, and shortened survival[1]
- Many 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[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 June 30, 2024, in real-world clinical settings
- Data were extracted from index date to the end of data availability, end of study (September 30, 2024), or death, whichever occurred first
- This analysis was conducted on a subset of patients with a PLT count <100 x 10^9/L at index who had data available for ≥90 days post-index
  - PLT response was defined per IWG criteria at any time following 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
  - Patients who achieved a PLT response within 90 days from index are reported on separately
- 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

References

1. Mascarenhas J, et al. *Expert Rev Hematol.* 2022;15:671-684. 2. Masarova L, et al. *Eur J Haematol.* 2018;100:257-263. 3. Masarova L, et al. *Leuk Res.* 2020;91:106338. 4. VONJO® (pacritinib) Capsules Prescribing Information. Waltham, MA: Sobi, Inc. 2024. 5. Vachhani P, et al. *Blood.* 2023; 142(Supplement 1):4554. 6. Marrone M, et al. *J Clin Onc.* 2024;42:6579-6579.

RESULTS

- 90 patients were included (PLT count <100 x 10^9/L at index and had data for ≥90 days post-index)
- Of the 90 patients, 47 (52.2%) met the criteria for PLT response (PLT-R), and 33 of the 47 (70.2%) achieved a response within 90 days
- A majority of patients with PLT-R by day 90 were male or White and had anemia at index with similar patient characteristic for those that did not achieve response (PLT-NR) by day 90 **(Table 1)**
- PLT-R had a higher median PLT count at index compared to PLT-NR **(Table 1)**

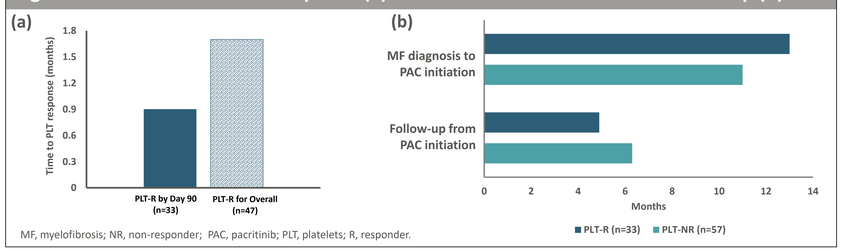
Table 1. Baseline treatment characteristics among patients with PLTL-R by day 90

	PLT-R (n=33)	PLT-NR (n=57)
Age at PAC initiation (index), years		
Median (Q1, Q3)	76 (71, 82)	76 (68, 80)
Sex, n (%)		
Male	22 (66.7)	34 (59.6)
Race, n (%)		
White	21 (63.6)	36 (63.2)
Other/Unknown	11 (33.3)	16 (28.1)
Ruxolitinib use prior to PAC, n (%)	16 (48.4)	26 (45.6)
PLT count at PAC initiation (index)		
Median (Q1, Q3)	64.5 (45.0, 81.0)	41 (23, 57)
Hb level at PAC initiation (index)		
Median (Q1, Q3)	8.8 (7.9, 10.1)	8.8 (7.5, 10.6)

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

- Median time to PLT response from index was 1.7 months overall (n=47) and 0.9 months for the 33 patients with a response by day 90 (**Figure 1a**)
- The median follow-up from index was longer in PLT-R (13 months) than PLT-NR (11 months) **(Figure 1b)**
- Median time from MF diagnosis to index was longer in PLT-NR (6.3 months) compared to PLT-R (4.9 months) (**Figure 1b**)

Figure 1. Median time to PLT response (a) and time to PAC initiation and follow-up (b)

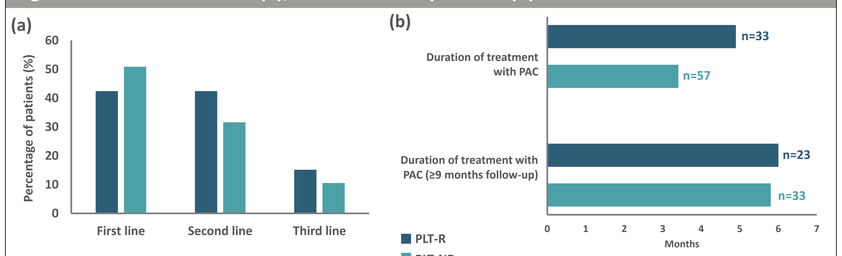


MF, myelofibrosis; NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Treatment patterns with pacritinib

- First line (42%) and second line (42%) PAC was similar in PLT-R **(Figure 2a)**
- Duration of PAC treatment in patients with ≥9 months follow-up was 6 months in PLT-R (n=23) and 5.8 months in PLT-NR (n=33) (**Figure 2b**)

Figure 2. Line of treatment (a), and treatment patterns (b)

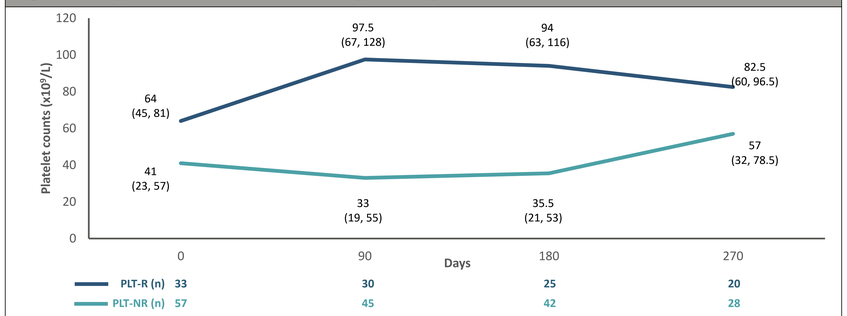


NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Platelet response with pacritinib treatment

- Median PLT count increased from index to day 90 by 58% in PLT-R, and remained stable through day 180 (41% median increase) and day 270 (32% median increase) (**Figure 3**)
- Median PLT count remained stable from index to day 180 (0% change) with a 14% median increase at day 270 in PLT-NR (**Figure 3**)

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

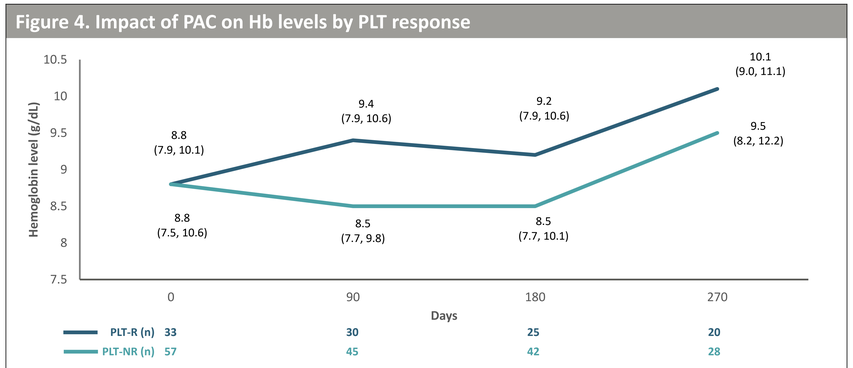


NR, non-responder; PAC, pacritinib; PLT, platelets; R, responder.

Hemoglobin response with pacritinib treatment

- Median Hb remained stable through day 180, and increased by a median of 4% and 2.8% from index to day 270 in PLT-R and PLT-NR respectively **(Figure 4)**

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



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

Overall Survival

- From day 90, 6-month survival probability was 80.8% (95% CI: 62.1, 90.9) in PLT-R and was similar in PLT-NR (80.7%; 95% CI: 66, 89.5) which also included 14 patients with a PLT response after day 90
- OS was consistent among PLT-R by day 90 and PLT-NR suggesting that longer follow-up may be required to delineate survival benefits for those with a PLT response including those with a response after day 90

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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For additional information contact: Michael Marrone : michael.marrone@sobi.com