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

Douglas Tremblay[1]; Abiola Oladapo[2]; Emily Levine[3]; Michael Marrone[2]; Djibril Liassou[3]; Purvi Suthar[2]; Adina Geprifti[3]; Gerard Hoehn[2]; Michael Vredenburg[2]; Bruce Feinberg[3]; Naveen Pemmaraju[4]; James Rossetti[5]

[1]Icahn School of Medicine at Mount Sinai, New York, United States of America; [2]Sobi Inc., Waltham, United States of America; [3]Cardinal Health, Dublin, United States of America; [4]University of Texas MD Anderson Cancer Center, Houston, United States of America; [5]University of Pittsburgh Medical Center, Pittsburgh, United States of America

Poster #PS1842

SUMMARY

- In real-world clinical settings, the majority of patients with myelofibrosis (MF) and platelet (PLT) counts ≥50 x10^9/L who were treated with pacritinib (PAC) and underwent baseline and day 180 spleen assessments showed a reduction in spleen size, and no increases in spleen size were observed. Stabilization or improvements in hemoglobin (Hb) and PLT levels were also observed.
- These results highlight PAC's potential therapeutic efficacy in patients with PLT ≥50 x10^9/L. Additional longitudinal follow-up in the real-world clinical setting would help to better understand the long-term benefits of PAC treatment in this patient population.

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 ≥50 x10^9/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 for PLT and Hb are 20,000/µL-100,000/µL with IWG PLT response and ≥1.0g/L Hb increase relative to baseline, respectively.
- 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], 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) JAK-inhibitor.
- At MF diagnosis, median age was 68 years (IQR: 62 to 74). Most patients were male (51.4%) and White (80%).
- 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

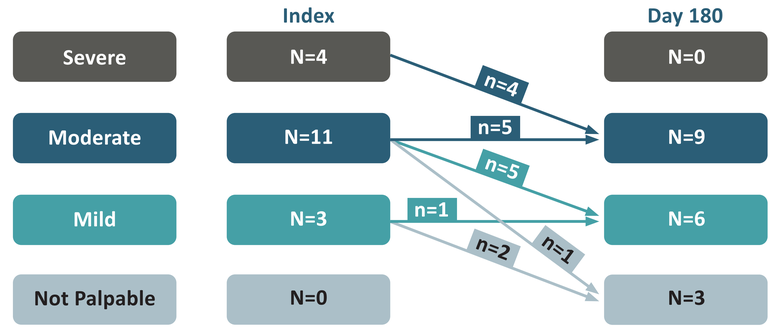
	PAC treated patients (n=35)
Age at PAC initiation (years)	
Median (Q1-Q3)	**70 (65.0-75.0)**
Gender at birth (n, %)	
Male	**18 (51.4)**
Race (n, %)*	
Asian	**2 (5.7)**
Black or African American	**4 (11.4)**
Native Hawaiian or Other Pacific Islander	**1 (2.9)**
White	**28 (80)**
Time from initial MF diagnosis 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
Abbreviations: MF=myelofibrosis; Q1-Q3=First quartile-third quartile

Spleen size reduction (Figure 1 and 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.

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



Note: 18 patients had spleen assessment at index and day 180; Spleen Size categories: Not Palpable [NP], minimally palpable <5 cm below costal margin; Mild: 5-10 cm palpable; Moderate: 11-20 cm palpable; and Severe: >20 cm palpable. 80% of patients were still on treatment at day 180. No patient had worsening of spleen category through both day 90 as well as day 180.

Table 2. Spleen Response

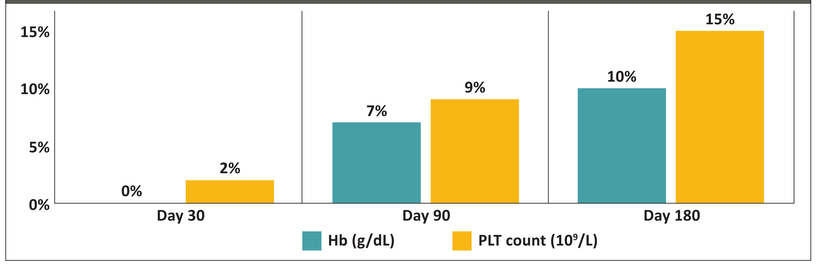
	PAC treated patients (n=35)	Pacritinib as 1st JAK inhibitor (n=18)	Pacritinib as 2nd JAK inhibitor (n=17)
Spleen size changes from index to day 180 post-index*			
n, % evaluated	**18 (51.4)**	**9 (50)**	**9 (53)**
No change in spleen size	**6 (33.3)**	**2 (22.2)**	**4 (44.4)**
Mild to non palpable	**2 (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

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) (**Table 3**).
  - 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**).

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



Hemoglobin

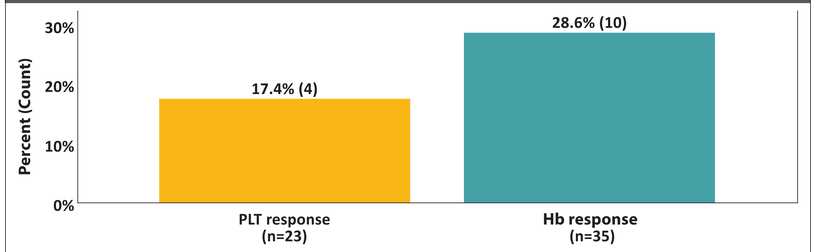
- Median Hb was 9.0 g/dL (IQR: 8.0 to 9.2) at index, and most patients had Hb <10 g/dL (85.7%; 30/35) (**Table 3**).
  - Increases in Hb were seen from index through day 180 [10% median increase (IQR: -1.1% to 12.5%)] (**Figure 2**).
  - By day 180, 28.6% (10/35) of patients achieved ≥1.0 g/dL increase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

Table 3. Hemoglobin and Platelet Labs

	PAC treated patients (n=35)	Pacritinib as 1st JAK inhibitor (n=18)	Pacritinib as 2nd JAK inhibitor (n=17)
Lab Values, closest to but prior to PAC initiation			
Hemoglobin (g/dL)			
Median, Q1-Q3	**9, 8.0-9.2**	**9, 8.0-9.7**	**8, 7.8-9.0**
Platelets (10^9/L)			
Median, Q1-Q3	**60, 1.0-110.0**	**54, 1.0-110.0**	**61, 55.0-88.0**
Percent change in Lab Values at day 180 vs index (%)			
Hemoglobin (g/dL)			
n, %	**29 (82.9)**	**18 (100)**	**11 (64.7)**
Median, Q1-Q3	**10, -1.1-12.5**	**2, -5.6-11.8**	**13, 9.9-13.3**
Platelets (x10^9/L)			
n, %	**29 (82.9)**	**18 (100)**	**11 (64.7)**
Median, Q1-Q3	**15, 6.8-30.0**	**9, -7.7-30.0**	**20, 14.1-36.7**

Abbreviations: g/dL=grams per deciliter; L=liter Q1-Q3=First quartile-third quartile

Figure 3. Percent of Patients with Notable Lab Value Improvement from Index to Day 180 Post-Index



Abbreviations: µl=microliter; HB=hemoglobin; IWG=International Working Group; L=liter; PLT=platelet

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 4**).

Figure 4. Overall Survival of Patients Treated with Pacritinib (Months)

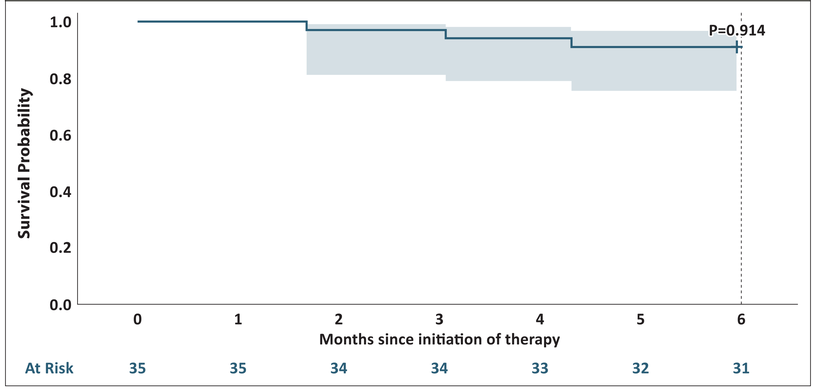


Table 4. Overall Survival

	PAC treated patients (n=35)	Pacritinib as 1st JAK inhibitor (n=18)	Pacritinib as 2nd JAK inhibitor (n=17)
OS from initiation of index (months)[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 180 days point estimate			
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.
Abbreviations: CI=confidence interval; KM=Kaplan-Meier; NE=not evaluable; NR=not reached; OS=overall survival

Study Limitations

- Patients lost to follow-up after at least 6 months of follow-up data from PAC initiation (except for cases of death) may differ from those who continued their care at the same medical center for 6 months or longer.
- 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

References

1. Mascarenhas J, Hoffman R, Talpaz M, Gerds AT, Stein B, Gupta V, et al. *Pacritinib vs Best Available Therapy, Including Ruxolitinib, in Patients With Myelofibrosis: A Randomized Clinical Trial*. JAMA Oncol. 2018;4(5):652-9.
2. Mesa RA, Vannucchi AM, Mead A, Egyed M, Szoke A, Suvorov A, et al. *Pacritinib versus best available therapy for the treatment of myelofibrosis irrespective of baseline cytopenias (PERSIST-1): an international, randomised, phase 3 trial.* Lancet Haematol. 2017;4(5):e225-e36.
3. Gerds AT, Savona MR, Scott BL, Talpaz M, Egyed M, Harrison CN, et al. *Determining the recommended dose of pacritinib: results from the PAC203 dose-finding trial in advanced myelofibrosis.* Blood Adv. 2020;4(22):5825-35.

Disclosures

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, Gerard Hoehn, and Michael Vredenburg are employees of Sobi Inc.

Acknowledgements

The authors thank Ryan Laughlin for poster development, Tammy Schuler, Puja Aggarwal, Caleb Paydar, Angele Kotomale, Camryn Craig, and Carol Toyoken for assistance with content development, and Kehinde Ogebule for project management, all employed by Cardinal Health. This poster was created by the authors in accordance with Good Publication Practice (GPP) 2022 guidelines (https://www.ismpp.org/gpp-2022). Sobi reviewed and provided feedback on the poster.
The authors had full editorial control of the poster and provided their final approval of all content.
This research is funded by Sobi Inc.

For additional information contact **Abiola Oladapo**: Abiola.Oladapo@sobi.com