Real-World Treatment Patterns and Outcomes in Patients with Myelofibrosis Treated with Pacritinib in the United States

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CONCLUSIONS

- **In addition to spleen and symptom benefits observed in previous clinical trials, real-world outcomes demonstrate stability or improvement in thrombocytopenia and/or anemia in patients with myelofibrosis treated with pacritinib**
- **Overall survival in the 1st line and 2nd line setting compare favorably with other JAK inhibitor historical controls**

INTRODUCTION

- Pacritinib, a JAK1 sparing JAK2/IRAK1/ACVR1 inhibitor, has shown clinically significant activity in spleen volume and symptom reduction in patients with thrombocytopenic myelofibrosis (MF)
- Thrombocytopenic MF is challenging to manage and patients with platelets (PLT) <50 x10^9/L have limited median overall survival (OS) of 15 months[1]
- Poor overall survival has also been reported in patients with MF and anemia[2]
- Since approval as the only agent for patients with MF and severe thrombocytopenia (PLT <50 x10^9/L), real-world evidence of pacritinib use is limited

OBJECTIVES

To assess clinical and demographic characteristics and real-world treatment patterns and outcomes in patients with MF treated with pacritinib in clinical settings in the US

METHODS

- For this retrospective observational study, Integra-PrecisionQ database, a de-identified harmonized dataset including electronic medical record (EMR) data and practice management data, was used to identify patients diagnosed with MF (ICD-10: 75.81, D47.4) treated with pacritinib between June 2022 and August 2023
- Descriptive statistics were used to characterize patient demographic and clinical characteristics, treatment patterns, and outcomes
- Treatment-related outcomes include change in PLT, hemoglobin (Hb) from pacritinib initiation (i.e., index) and 30-day intervals post-index
- Overall survival was assessed from the time of pacritinib initiation through the end of the observation period (October 2023), and survival probabilities and corresponding 95% confidence intervals (CI) estimated using the Kaplan Meier method

Disclosures

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RESULTS

- Overall, 142 patients were treated with pacritinib during the study period contributing a median follow-up of 6 months from index (Interquartile range [IQR:] 4 to 11 months)
- Of the 119 patients with complete lab values at index and ≥1 during follow-up:
  - 28.5% (34/119) had severe thrombocytopenia (PLT<50 x10^9/L) at index
  - 29% (35/119) had severe anemia (Hb <8.0 g/dL) at index
- A majority of patients were male (60%) or White (66%)
- Median age at MF diagnosis was 72 years (IQR: 64 to 79), and the median time from MF diagnosis to pacritinib initiation was 13.4 months (IRQ: 0.6 to 49.6)
- MF-related line of therapy overall and by baseline PLT and Hb levels are presented in **Figure 1**
- Median time from pacritinib initiation through last dose or end of observation period was 5.3 months (IQR: 2.4 to 9.1) overall (n=142) (**Table 1**)
  - Among patients with ≥6 months of follow-up after starting pacritinib (n=78) median duration of pacritinib treatment was 8.5 months (IQR: 5.9 to 11.2)

Figure 1: Pacritinib treatment by line of therapy (LOT) and PLT (x10^9/L) and Hb (g/dL) values

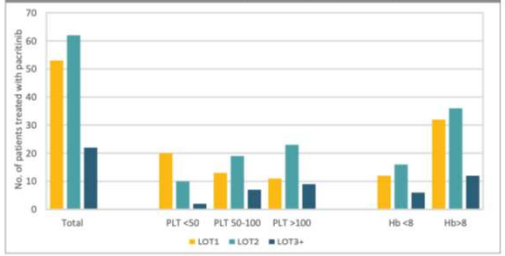


Table 1: Time to pacritinib initiation and duration of use by baseline PLT (x10^9/L) and Hb (g/dL) values

	Time to pacritinib initiation from MF diagnosis (months)		Duration of treatment with pacritinib (months)*			
			Overall		≥6-months follow-up	
	No.	Median (IQR)	No.	Median (IQR)	No.	Median (IQR)
Overall	142	13.4 (0.6 to 49.6)	142	5.3 (2.4 to 9.1)	78	8.5 (5.9 to 11.2)
PLT <50	34	1.7 (0.0 to 17.8)	34	5.0 (2.0 to 7.6)	18	7.5 (5.9 to 10.8)
PLT 50-100	40	21.2 (1.0 to 48.7)	40	5.4 (2.7 to 9.6)	18	10.3 (6.9 to 13.0)
PLT >100	45	15.4 (5.6 to 58.3)	45	4.9 (2.3 to 9.0)	26	8.1 (7.0 to 10.7)
Hb <8	35	8.4 (1.0 to 50.5)	35	4.3 (1.9 to 7.9)	16	9.3 (6.8 to 11.9)
Hb >8	84	14.0 (0.5 to 50.5)	84	5.5 (2.4 to 9.0)	46	8.8 (6.0 to 11.2)

*Including patients currently treated with pacritinib through last dose or end of the study period

Pacritinib treatment and hematologic outcomes

- Median PLT counts in patients with PLT <100 at index demonstrated an early increase within 30 days that was sustained throughout the observation period (**Figure 2**)
- An early increase in median Hb was noted and sustained throughout the observation period, with a more profound increase of nearly 1 g/dL by day 30 in patients with Hb <8.0 g/dL at index (**Figure 3**)
- Among patients treated with ruxolitinib prior to pacrtitinib – PLT and Hb demonstrated a consistent increase from pacritinib initiation through the end of the observation period (**Table 2**)

Figure 2: Median PLT values (x10^9/L) by baseline category over follow-up

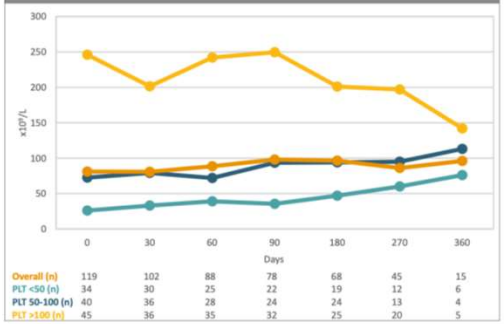


Figure 3: Median Hb values (g/dL) by baseline category over follow-up

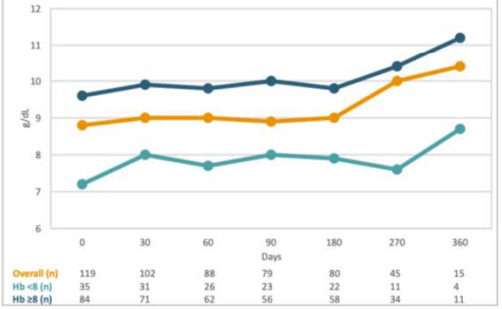


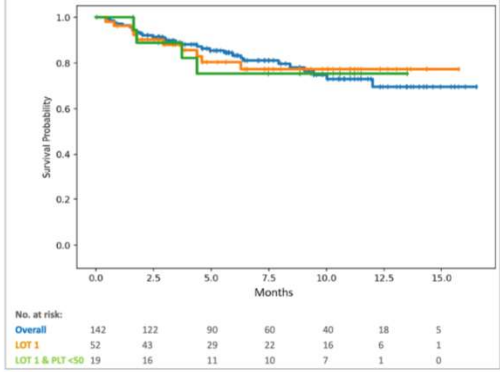
Table 2. PLT and Hb values among patients treated with ruxolitinib prior to pacritinib

	Index	Post-Index Day					
		30	60	90	180	270	360
PLT (x10^9/L)							
No. of patients	69	58	51	43	41	25	7
Median	91.0	90.0	97.0	107.0	94.0	97.0	97.0
Hb (g/dL)							
No. of patients	69	58	51	44	47	25	7
Median	8.7	9.0	9.0	9.0	9.0	10.0	10.4

Pacritinib treatment and 12-month overall survival

- The 12-month overall survival probability following initiation of pacritinib was 69.4% (95% CI: 56.8 to 79.0) for the overall MF population (n=142) (**Figure 4**). This compares favorably with survival previously report in JAK inhibitor historic controls[1]
- Overall survival probability was 77.3% (95% CI: 61.5 to 87.3) for patients treated with first-line pacritinib (n=52), and 75.2% (95% CI: 46.3 to 90.0) for those with PLT <50 in the first-line (n=19)
- Among the 59 patients treated with pacritinib in the second-line, 12-month overall survival was 72.1% (95% CI: 42.4 to 88.2)
- Among patients treated with ruxolitinib prior to pacritinib (n=84), overall survival was 65.3% (95% CI: 47.2 to 78.5)

Figure 4. Pacritinib treatment and overall survival (June 2022 - October 2023)



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