

Real-World Effectiveness of Pacritinib in Patients with Myelofibrosis Who Have Thrombocytopenia And Anemia

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CONCLUSIONS

- Real-world data in patients with myelofibrosis (MF) and bicytopenia (thrombocytopenia and anemia) show hematologic improvement or stability with pacritinib (PAC)
- Overall survival (OS) and 12-month survival probability in this subgroup of patients with a poor prognosis, were comparable with prior PAC-treated cohort findings
- These findings support clinical utility of PAC in addressing unmet needs of patients with MF and bicytopenia

BACKGROUND

- MF is a rare myeloproliferative neoplasm characterized by anemia, thrombocytopenia, splenomegaly, potential for leukemic progression, and shortened survival¹
- The occurrence of bicytopenia in patients with MF is a therapeutic challenge due to treatment-related myelosuppression caused by Janus kinase (JAK)1/2 inhibitors (ruxolitinib and fedratinib)²
- 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^{1,3}
- In the phase 3 PERSIST-2 trial (NCT02055781), treatment with PAC was associated with spleen volume reduction, symptom benefit, and reduced transfusions in patients with bicytopenia⁴

AIM

To evaluate treatment patterns, hematologic outcomes, and survival in real-world clinical practice among PAC-treated patients with MF presenting with bicytopenia

METHODS

- Integra-PrecisionQ database, including electronic health and practice management data (80% community oncology practices) was used to identify patients with MF (based on International Classification of Disease, Tenth Revision [ICD-10] diagnostic codes: D47.4, D75.81, and D47.1) who initiated treatment with PAC (index) between June 1, 2022, and June 30, 2024, in real-world clinical settings
- Data were extracted from the index date to the end of data availability, end of study (September 30, 2024), or death, whichever occurred first
- Treatment patterns and outcomes were analyzed in a subset of patients with MF and bicytopenia (hemoglobin [Hb] <8 g/dL and a platelet [PLT] count <100 × 10⁹/L) or severe bicytopenia (Hb <8 g/dL and a PLT count <50 × 10⁹/L)
- Treatment-related outcomes assessed included:
 - PLT and Hb levels at 30-day intervals through the end of the study period
 - OS and 12-month survival probabilities were estimated using the Kaplan-Meier method
- Continuous variables were summarized using median and interquartile range (IQR), and categorical variables were described using counts and percentages

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RESULTS

- Of 179 patients treated with PAC and with laboratory data available at index and follow-up, 21.2% of patients had bicytopenia (n = 38) and 10.6% had severe bicytopenia (n = 19) at index
- Most patients with bicytopenia were male (63.2%) with a median (IQR) age at MF diagnosis of 74 years (67–80; **Table 1**)
- Median (IQR) time from MF diagnosis to index was 3.9 (0.1, 16.6) months (**Table 1**)
- Median (IQR) PLT count was 49.5 × 10⁹/L (27.0, 70.7) at index (**Table 1**)

Table 1. Baseline demographics and treatment characteristics of patients with bicytopenia (n = 38)	
Age at MF diagnosis, years	
Median (Q1, Q3)	74 (67, 80)
Sex, n (%)	
Male	24 (63.2)
Race, n (%)	
White	24 (63.2)
Other/unknown	10 (26.3)
Follow-up from MF diagnosis, months	
Median (Q1, Q3)	17 (6.0, 27.0)
Time from MF diagnosis to PAC initiation (index), months	
Median (Q1, Q3)	3.9 (0.1, 16.6)
Age at PAC initiation (index), years	
Median (Q1, Q3)	76 (69, 82)
Follow-up from PAC initiation (index), months	
Median (Q1, Q3)	8.0 (4.0, 15.0)
PLT count at PAC initiation (index), × 10⁹/L	
Median (Q1, Q3)	49.5 (27.0, 70.7)
Hb level at PAC initiation (index), g/dL	
Median (Q1, Q3)	7.2 (6.9, 7.5)

Hb, hemoglobin; MF, myelofibrosis; PAC, pacritinib; PLT, platelet; Q1, first quartile; Q3, third quartile.

Treatment patterns with PAC

- PAC was the most common first- and second-line treatment in patients with bicytopenia (**Table 2**)
- Median (IQR) time from MF diagnosis to first-line PAC initiation was 1.5 (0, 21.3) days (**Table 3**)

Table 2. Treatment patterns in patients with bicytopenia treated with PAC (n = 38)	
Treatment patterns	n (%)
1L PAC	19 (50)
2L PAC	15 (39.5)
3L+ PAC	5 (13.2)
1L treatment pattern in patients with 2L PAC	n (%)
Ruxolitinib	12 (80)
Pacritinib	1 (6.7)
Thalidomide	1 (6.7)
Hydroxyurea	1 (6.7)

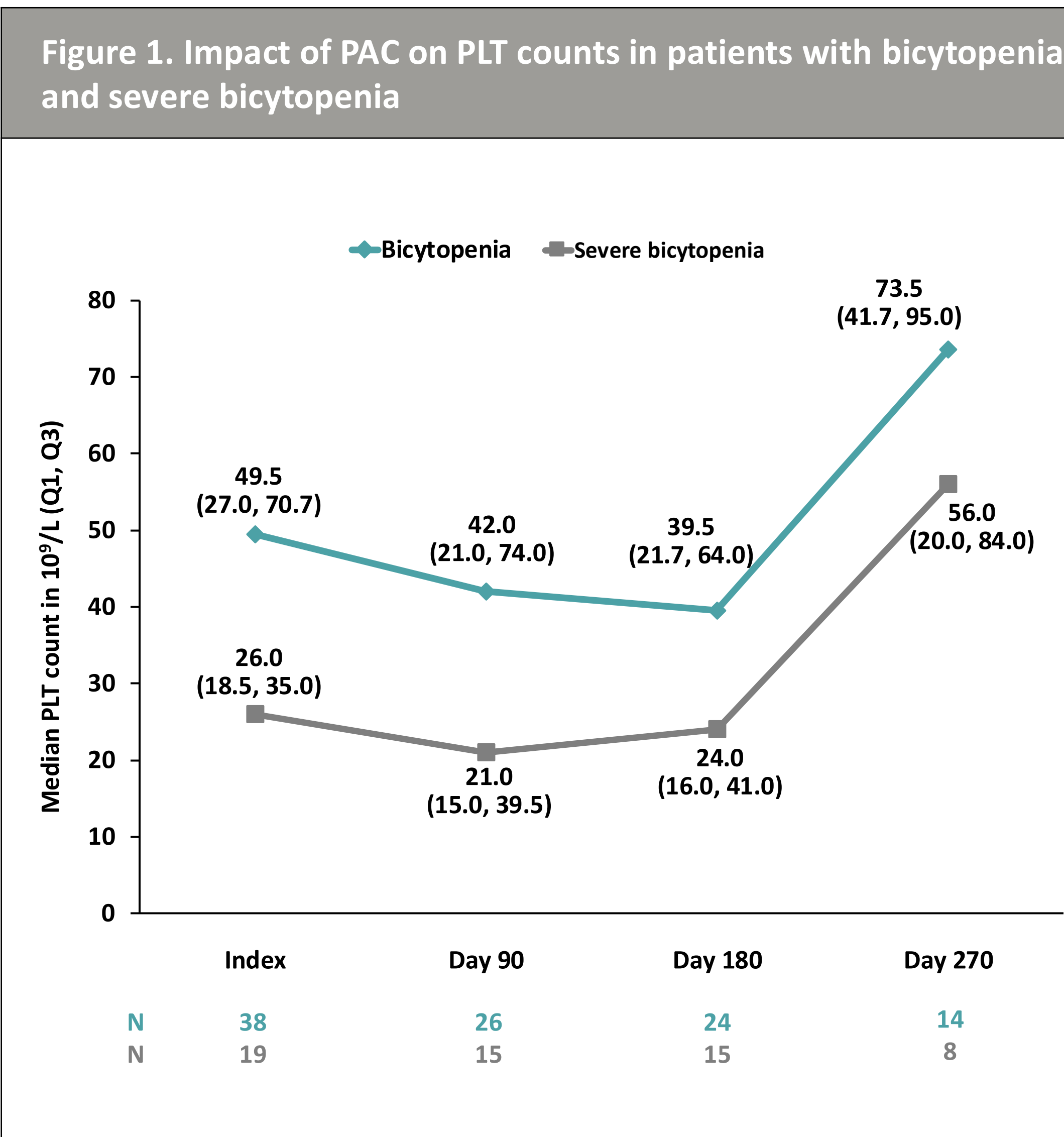
Patient treated with PAC in both 1L and 2L received an additional therapy alongside PAC in 2L. 1L, first-line; 2L, second-line; 3L+, third-line or later; PAC, pacritinib.

Table 3. Time to initiation and duration of PAC treatment in patients with bicytopenia (n = 38)	
Time from MF diagnosis to 1L PAC initiation, days (n = 19)	
Median (Q1, Q3)	1.5 (0, 21.3)
Time between end of previous MF therapy and 2L PAC initiation, days (n = 15)	
Median (Q1, Q3)	1.0 (1.0, 45.7)
Duration of PAC treatment, (≥9 months follow-up), months (n = 17)	
Median (Q1, Q3)	5.0 (3.9, 10.8)

1L, first-line; 2L, second-line; MF, myelofibrosis; PAC, pacritinib; PLT, platelet; Q1, first quartile; Q3, third quartile.

PLT response with PAC treatment

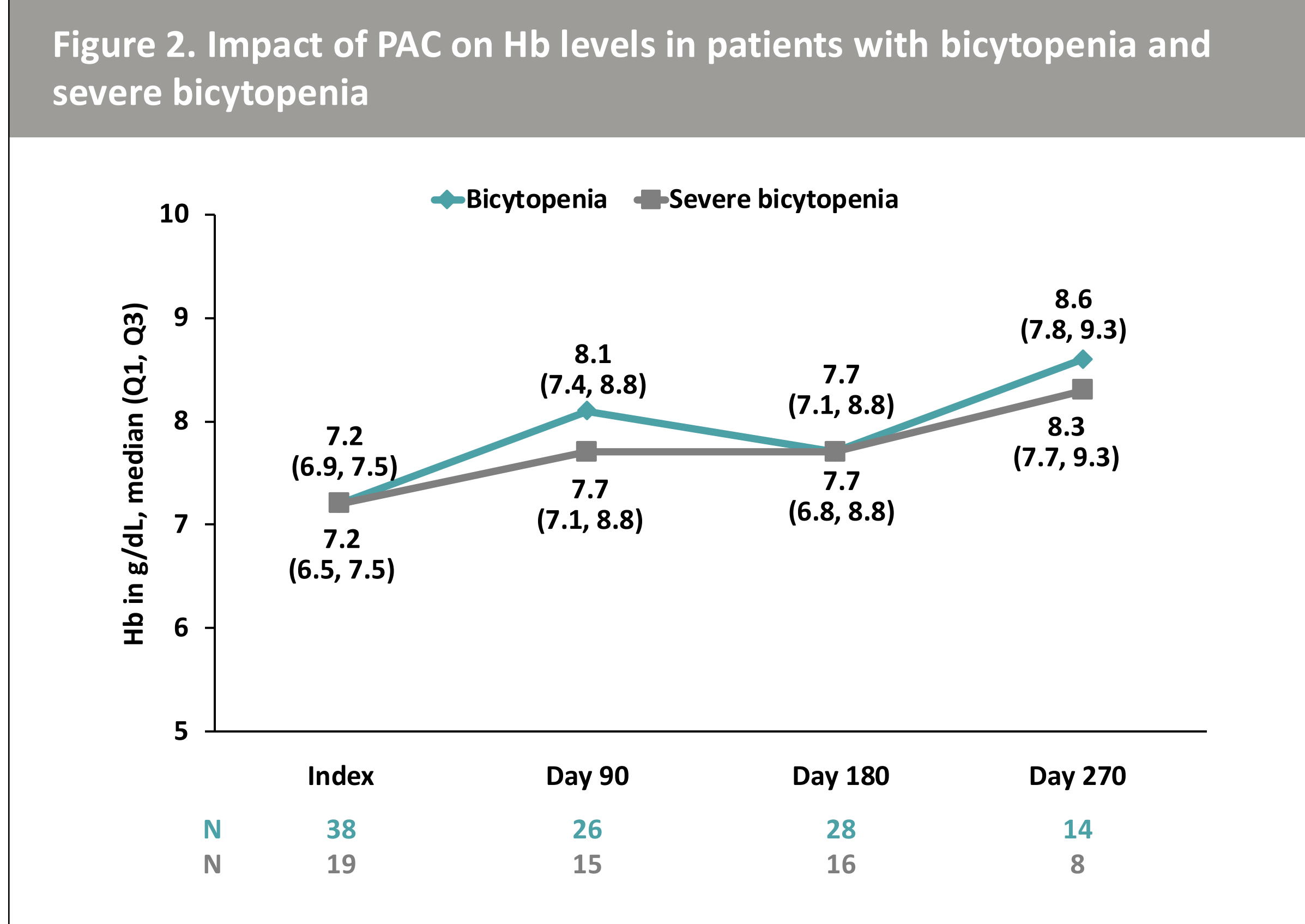
- In patients with bicytopenia, the median PLT count remained relatively stable from index with improvements observed at post-index day 270 (**Figure 1**)
 - A similar pattern in median PLT count was observed in patients with severe bicytopenia (**Figure 1**)



PAC, pacritinib; PLT, platelet; Q1, first quartile; Q3, third quartile.

Hb response with PAC treatment

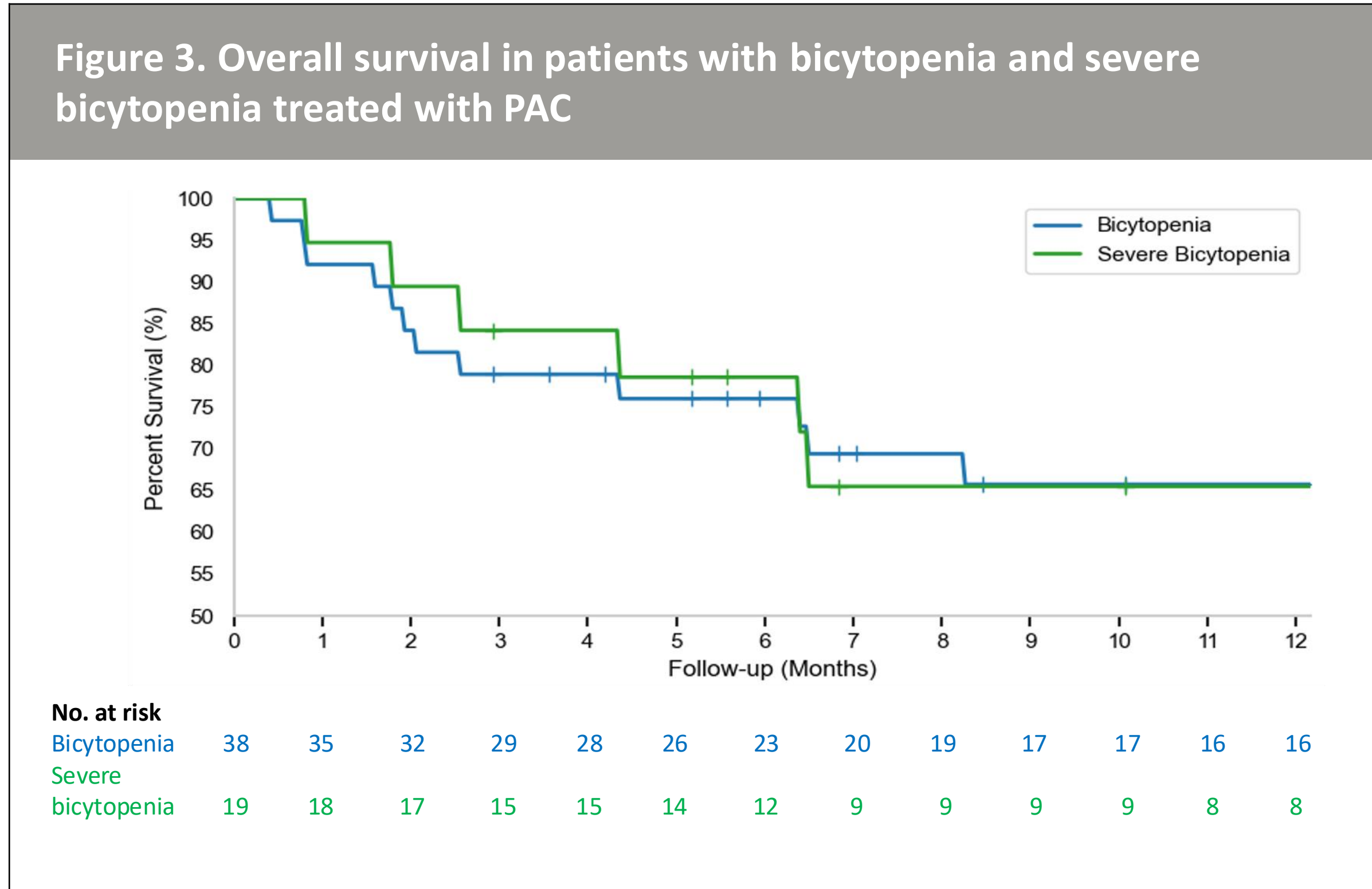
- In patients with bicytopenia, the median Hb level increased from index and improved by almost 1 g/dL by post-index day 90 and continued to improve through post-index day 270 (**Figure 2**)
 - A similar pattern in median Hb level increase was observed in patients with severe bicytopenia (**Figure 2**)



Hb, hemoglobin; PAC, pacritinib; Q1, first quartile; Q3, third quartile.

Overall survival

- The 12-month survival probability was 65.8% (95% CI: 47.2, 79.1) for patients with bicytopenia and 65.5% (95% CI: 38.3, 83.0) for patients with severe bicytopenia (**Figure 3**)



PAC, pacritinib.

Limitations

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

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