Transfusion-Related Cost and Time Burden Offsets in Patients With Myelofibrosis Treated With Pacritinib Compared to Best Available Therapy Based on PERSIST-2 Trial

PF1306

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CONCLUSIONS

The reduction in transfusion rates associated with pacritinib (PAC) treatment relative to best available treatment (BAT) is projected to decrease transfusion-related medical costs and time burden for patients with cytopenic myelofibrosis (MF)

BACKGROUND

- Anemia (hemoglobin <10 g/dL) is a key clinical feature of MF, a rare myeloproliferative neoplasm[1]
- Anemia is associated with significant disease burden, particularly in patients dependent on red blood cell (RBC) transfusions for management, as it negatively impacts their quality of life and disease prognosis[1,2-4]
- In the PERSIST-2 trial (NCT02055781), treatment with PAC (a JAK1-sparing inhibitor of JAK2/IRAK1/ACVR1) was associated with anemia benefit[5]
- A significantly higher proportion of patients who were non-transfusion independent (non-TI) at baseline achieved TI when treated with PAC vs BAT (37% vs 7%) in any 12 weeks over a 24-week interval[5]
- A significantly higher proportion of patients had a ≥50% reduction in transfusion burden with PAC than with BAT (49% vs 9%) with lower RBC transfusion rates (mean: 2.45 vs 3.54 per 30-day period)[5,6]

AIM

To estimate the projected differences in transfusion-related cost and time burden associated with PAC vs BAT treatment from a US payer perspective

METHODS

- An economic evaluation was conducted based on transfusion-related data from the PERSIST-2 trial for patients treated with PAC or BAT (including ruxolitinib [RUX] and erythroid support [ES]) who enrolled for ≥12 weeks before study termination[5,6]
- Transfusion status (TI and non-TI) at baseline (ie, initiation of PAC or BAT) and over any 12-week interval within the 24-week study period was defined based on Gale criteria[7] (ie, presence or absence of RBC transfusions; **Table 1**)
- Mean RBC transfusion rates over a 30-day period, including all reported transfusions within the initial 24-week study period, were annualized and used as proxy for transfusion-related visits (**Table 1**)[6]
- Annual transfusion-related cost estimates by transfusion status were based on a previous MF burden of illness study, which utilized IBM MarketScan data[8] and was adjusted to 2024 US dollars using the medical component of the Consumer Price Index[9]
  - Projected medical costs for PAC and BAT were calculated by multiplying the cost estimates with the proportion of patients with non-TI or TI status in each group over any 12-week interval within the 24-week study period[5,6]
- Transfusion-related time burden estimates were based on previously reported RBC transfusion visits in transfusion dependent patients with -thalassemia[10]
  - Projected transfusion-related time burden for PAC and BAT was calculated by multiplying the estimated time spent on average per transfusion visit with the average RBC transfusion rates per-patient per-year within the PAC and BAT arms[6,10]
- Projected cost differences and time savings were calculated as the difference between PAC and BAT for the projected cost and time burden estimates, respectively

Table 1. Model inputs

	Overall		PLT <50 × 10⁹/L[a]		PLT ≥50 × 10⁹/L[a]	
	PAC	BAT	PAC	BAT	PAC	BAT
Transfusion status (baseline)						
Non-TI	41	43	25	26	16	17
TI[b]	51	45	16	12	34	32
Total	92	88	41	38	50	49
Proportion of patients who achieved TI status[c]						
Number of patients (%)	15/41 (36.6)	3/43 (6.9)	7/25 (28.0)	2/26 (7.7)	8/16 (50.0)	1/17 (5.9)
Proportion of patients who maintained TI status[d]						
Number of patients (%)	42/50 (84.0)	40/45 (88.9)	12/16 (75.0)	10/12 (83.3)	29/33[e] (87.9)	29/32 (90.6)
RBCT rates over 30-day period						
Non-TI, mean (±SE)	2.45 (0.49)	3.54 (0.44)	3.33 (0.77)	4.00 (0.62)	1.47 (0.45)	3.01 (0.61)
TI, mean (±SE)	0.26 (0.11)	0.09 (0.04)	0.36 (0.15)	0.13 (0.13)	0.22 (0.15)	0.08 (0.04)

[a]Platelet categories at baseline (ie, treatment initiation with PAC or BAT) in the PERSIST-2 trial.
[b]Two patients had missing Day 1 PLT information and could not be classified into subgroups.
[c]Patients with non-TI status at baseline who achieved TI status during the 24-week study period.
[d]Patients with TI status at baseline who maintained TI status during the 24-week study period.
[e]One patient with TI status at baseline had a missing transfusion log and status could not be determined.
BAT, best available treatment; non-TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; RBCT, red blood cell transfusion; SE, Standard error; TI, transfusion independent.

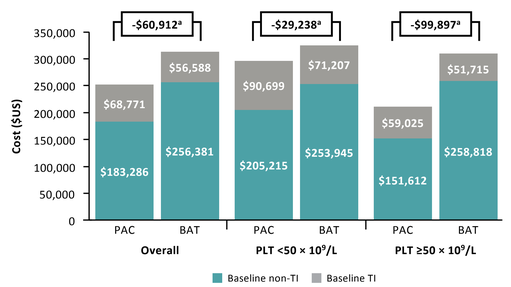
RESULTS

PAC reduced transfusion-related projected medical costs

Overall, the annual transfusion-related cost with PAC was projected to be 19.5% lower than with BAT, with a cost saving of $60,912 per patient compared with BAT (Figure 1)

- Among patients who were non-TI at baseline, projected annual cost saving per patient for PAC vs BAT was $73,095 (**Figure 1**)

Figure 1. Annual transfusion-related medical cost per patient (USD)



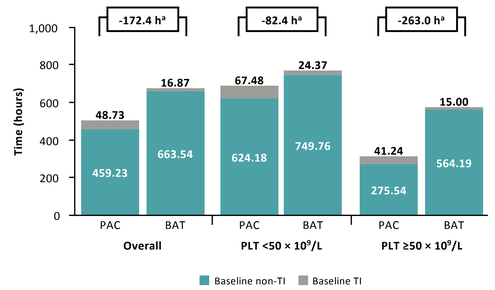
[a]Cost burden for PAC minus cost burden for BAT. Differences <0 indicate cost savings for PAC relative to BAT; differences >0 indicate cost savings for BAT relative to PAC. BAT, best available treatment; non-TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; TI, transfusion independent; USD, United States dollar.

PAC reduced transfusion-related projected time burden

Annual transfusion-related time burden with PAC was projected to be 25.3% lower than with BAT, with a time saving per patient of 172.4 hours compared with BAT (PAC: 507.9 hours vs BAT: 680.4 hours), primarily driven by RBC transfusion procedure/recovery time (Figures 2 and 3)

- Among patients who were non-TI at baseline, projected annual time savings per patient for PAC vs BAT was 204.3 hours (**Figure 2**)

Figure 2. Annual transfusion-related time burden per patient (h)

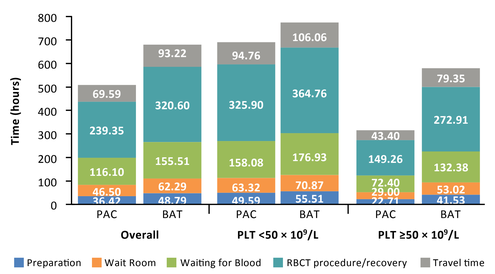


[a]Time burden for PAC minus time burden for BAT. Differences <0 indicate time savings for PAC relative to BAT; differences >0 indicate time savings for BAT relative to PAC. BAT, best available treatment; h, hours; non -TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; TI, transfusion independent.

Results remained robust regardless of baseline PLT count

- Annual transfusion-related cost saving per patient with PAC compared with BAT was $29,238 and $99,897 in patients with baseline PLT <50 × 10⁹/L and PLT ≥50 × 10⁹/L, respectively (**Figure 1**)
- Annual transfusion-related time saving per patient with PAC compared with BAT was 82.4 and 263.0 hours in patients with baseline PLT <50 × 10⁹/L and PLT ≥50 × 10⁹/L, respectively (**Figure 2**)
- Higher projected cost and time savings for PAC vs BAT were observed among patients with PLT ≥50 × 10⁹/L (**Figures 1 and 2**)

Figure 3. Annual transfusion-related time burden by activity per patient (h)

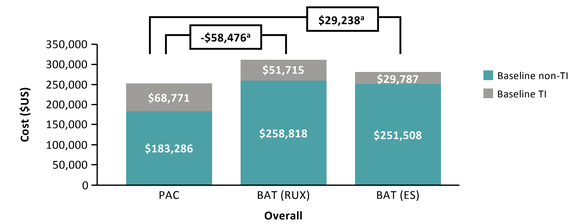


BAT, best available treatment; h, hours; PAC, pacritinib; PLT, platelet; RBCT, red blood cell transfusion.

Results remained robust regardless of type of BAT utilized

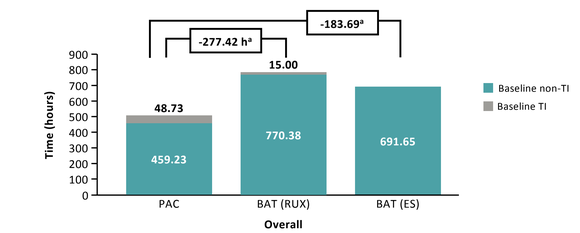
- Annual transfusion-related cost savings per patient with PAC was $58,476 and $29,238 compared with RUX and ES, respectively (**Figure 4**)
- Annual time saving per patient with PAC was 277.4 hours and 183.6 hours compared with RUX and ES, respectively (**Figure 5**)

Figure 4. Annual transfusion-related medical cost per patient (USD)



[a]Cost burden for PAC minus cost burden for RUX or ES. Differences <0 indicate cost savings for PAC relative to RUX or ES; differences >0 indicate cost savings for RUX or ES relative to PAC. BAT, best available treatment; ES, erythroid support; non-TI, non-transfusion independent; PAC, pacritinib; RUX, ruxolitinib; TI, transfusion independent; USD, United States dollar.

Figure 5. Annual transfusion-related time burden per patient (h)



[a]Time burden for PAC minus time burden for RUX or ES. Differences <0 indicate time savings for PAC relative to RUX or ES; differences >0 indicate time savings for RUX or ES relative to PAC. BAT, best available treatment; ES, erythroid support; non-TI, non-transfusion independent; PAC, pacritinib; RUX, ruxolitinib; TI, transfusion independent.

LIMITATIONS

- The current study estimated projected cost and time burden savings from a US perspective. Additional analyses may be warranted to determine potential impacts in other regions
- This analysis was based on data from a 24-week study period from the PERSIST-2 trial; future analysis utilizing data from real-world clinical settings over a longer period beyond this time point may be required to evaluate long-term benefits
- Projected cost savings were from a commercial payer perspective; future evaluations that incorporate the provider and patient's quality of life evaluation will be important to further describe the potential impact of PAC

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