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

Abiola Oladapo[1], Karisse Roman-Torres[1], Aaron Thomas Gerds[2], Stephen Oh[3]

[1]Sobi, Inc., Waltham, MA, USA; [2]Cleveland Clinic Taussig Cancer Institute, Cleveland, OH, USA; [3]Division of Hematology, Department of Medicine, Washington University School of Medicine, St. Louis, MO, USA.

CONCLUSIONS

The reduction in transfusion rates associated with pacritinib (PAC) treatment relative to best available therapy (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 transfusion independence 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 RBC transfusion-related data from the PERSIST-2 trial for patients treated with PAC or BAT (including ruxolitinib [RUX] and hematologic support therapies such as erythropoiesis-stimulating [ES] agents) 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 β-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 × 10^9/L[a]		PLT ≥50 × 10^9/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 therapy; non-TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; RBCT, red blood cell transfusion; SE, Standard error; TI, transfusion independent.

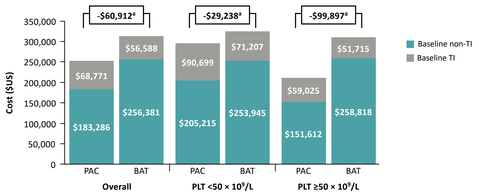
RESULTS

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)



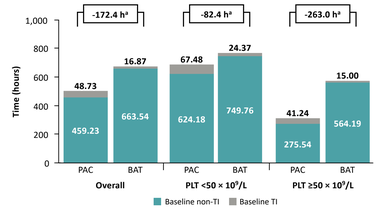
[a]Cost burden for PAC minus cost burden for BAT. Differences <0 indicate cost savings for PAC relative to BAT; differences >0 indicate cost savings for BAT relative to PAC. BAT, best available therapy; non-TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; TI, transfusion independent;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

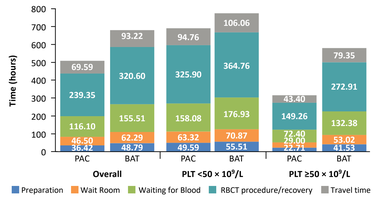


[a]Time burden for PAC minus time burden for BAT. Differences <0 indicate time savings for PAC relative to BAT; differences >0 indicate time savings for BAT relative to PAC. BAT, best available therapy; h, hours; non-TI, non-transfusion independent; PAC, pacritinib; PLT, platelet; TI, transfusion independent.

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 × 10^9/L and PLT ≥50 × 10^9/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 × 10^9/L and PLT ≥50 × 10^9/L, respectively (**Figure 2**)
- Higher projected cost and time savings for PAC vs BAT were observed among patients with PLT ≥50 × 10^9/L (**Figures 1 and 2**)

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

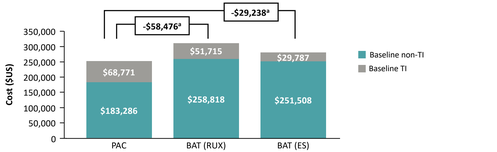


BAT, best available therapy; h, hours; PAC, pacritinib; PLT, platelet; RBCT, red blood cell transfusion.

Results remained robust regardless of type of BAT utilized

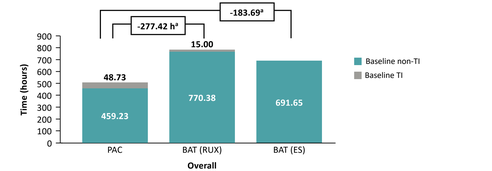
- Annual transfusion-related cost savings per patient with PAC was $58,476 and $29,238 compared with RUX and ES agents, respectively (**Figure 4**)
- Annual time saving per patient with PAC was 277.4 hours and 183.6 hours compared with RUX and ES agents, respectively (**Figure 5**)

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 therapy; ES, erythropoiesis-stimulating; 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 therapy; ES, erythropoiesis-stimulating; 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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For additional information contact: abiola.oladapo@sobi.com