Transfusion-related cost and time burden offsets in patients with myelofibrosis treated with momelotinib vs best available therapy

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Introduction

- Myelofibrosis (MF) is a myeloproliferative neoplasm characterized by anemia, splenomegaly, debilitating symptoms, and bone marrow fibrosis[1,2]
  - More than one-third of patients with MF present with anemia at diagnosis, with nearly all patients developing anemia over time[1]
- Currently approved Janus kinase (JAK) inhibitors may induce or worsen anemia, and management strategies for patients with anemia have limited effectiveness[3]
- Transfusion-dependent anemia is present in approximately one-quarter of patients at diagnosis; more patients require transfusions during the disease course, which is consistent with increasing rates of anemia[1,3]
- In addition to a negative impact on patients' quality of life, transfusion dependence (TD) imposes a high cost burden in MF, with annual total medical costs up to 9 times higher than those for patients without TD[3,4]
- Momelotinib is a JAK1/JAK2/activin A receptor type 1 (ACVR1) inhibitor that has demonstrated improvement in anemia and TD, in addition to symptom and spleen responses in patients with MF across 3 phase 3 trials, including SIMPLIFY-2 (NCT02101268)[5-7]
- SIMPLIFY-2 was a randomized, phase 3 trial that evaluated momelotinib vs best available therapy (BAT; 88.5% of patients treated with ruxolitinib) in ruxolitinib-experienced patients with MF. Fewer patients treated with momelotinib were transfusion dependent at week 24 (50% vs 64%) and a higher proportion of patients became transfusion independent (43% vs 21%) than those who received BAT[5]
- This analysis aimed to evaluate projected costs of transfusion-related medical care and outpatient visits and time burden for ruxolitinib-experienced patients with MF treated with momelotinib vs BAT

Aim

- To estimate projected differences between momelotinib and BAT in medical costs and transfusion-related costs and time burden

Methods

- Transfusion-related cost and time differences were calculated based on week 24 transfusion data from SIMPLIFY-2 and cost estimates derived from 2 commercial claims databases (**Figure 1**)
  - Transfusion rates and cost estimates were stratified by transfusion status (TD or transfusion independence [TI]/transfusion requiring [TR]), per the criteria shown in **Table 1**
  - Projected cost differences for medical care and outpatient transfusion visits for patients with MF were calculated using cost estimates based on analyses from the US claims IBM MarketScan Commercial database for the overall study population and the Medicare fee-for-service database for patients aged ≥65 years (**Table 2**) and applied to the SIMPLIFY-2 patient population
  - Projected time savings were based on transfusion rates calculated from SIMPLIFY-2, and time burden estimates were based on literature in patients with transfusion-dependent β-thalassemia (**Table 3**)[8]
- The mean number of visits for red blood cell (RBC) transfusions was reported in the Clinical Study Report for the overall population. For patients aged ≥65 years, this was calculated using individual patient-level data from SIMPLIFY-2. Data were stratified by treatment arm and baseline transfusion status (TD or TI/TR). Rates of RBC transfusion visits observed over the 24-week trial period in each group were re-scaled to reflect annualized rates

Methods (cont)

Figure 1. Method for Estimating Differences in Transfusion-Related Cost and Time Burden for Momelotinib vs BAT

Difference in total medical costs	=	Medical costs for TD vs TI/TR status	From analyses of IBM MarketScan Commercial database or Medicare (age ≥65 years) database	X	Differences in rates of TD vs TI/TR status for momelotinib vs BAT
Difference in outpatient transfusion visit costs	=	Cost per outpatient transfusion visit	From analyses of IBM MarketScan Commercial database or Medicare (age ≥65 years) database	X	Differences in rates of transfusion visits for momelotinib vs BAT
Differences in transfusion-related time burden	=	Time spent during each transfusion visit	From a survey of patients with transfusion-dependent β-thalassemia[8]	X	Differences in rates of transfusion visits for momelotinib vs BAT

From post hoc analyses of SIMPLIFY-2

BAT, best available therapy; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.

Table 1. Criteria for Transfusion Status

	SIMPLIFY-2	IBM MarketScan Commercial analysis	Medicare fee-for-service analysis
TD	≥4 RBC units transfused or a hemoglobin level <8 g/dL in the prior 8 weeks	≥2 RBC transfusions within any 4-week period in the prior 2 years	≥6 units of RBC transfusions or whole blood transfusions in any 12-week period
TI	No RBC transfusions and all hemoglobin levels ≥8 g/dL in the prior 12 weeks	No RBC transfusions in the prior 2 years	No RBC transfusions during the 180 days following the earliest confirmed diagnosis of anemia
TR	Does not meet criteria for TD or TI	Does not meet criteria for TD or TI	Does not meet criteria for TD or TI

Definitions were assumed as being functionally similar, as each study defines TI status as zero RBC transfusions and TD status as ≈0.5-unit transfusion per week.
RBC, red blood cell; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.

Table 2. Estimated Costs of Care[4]

Outcome	TI/TR[a]	TD[a]	All patients
IBM MarketScan Commercial Database Analysis[b]			
Total per-person per-year medical cost, US $	27,789	255,190	–
Outpatient transfusion visit cost, US $[c]	–	–	3790
Medicare Database Analysis[d]			
Total per-person per-year medical cost, US $	TI: 69,961 TR: 133,561	136,047	98,223
Outpatient transfusion visit cost, US $[c]	–	–	3790

TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.
[a] As defined in previous studies of IBM MarketScan Commercial or Medicare databases (**Table 1**).
[b] Estimates were based on JAK inhibitor-naive and JAK inhibitor-experienced patients.
[c] It was assumed that the mean cost per transfusion was equivalent to the mean cost for an outpatient transfusion claim.
[d] Estimates were available for JAK inhibitor-naive patients only. Costs for transfusion visits were not available from the Medicare database. It was, therefore, assumed that the mean cost per transfusion for Medicare was the same as in the IBM MarketScan Commercial database.

Table 3. Patient Time Burden Associated With RBC Transfusions[8]

Outcomes	Average time spent per 1 RBC transfusion procedure, hours	
	Mean	SD
Preparations	1.12	2.69
Waiting room	1.43	5.88
Waiting for blood	3.57	7.60
RBC transfusion procedure and recovery	7.36	11.26
Travel	2.14	4.16
Total time	15.62	23.24

RBC, red blood cell.

Results

Transfusion Status at Baseline and Week 24 (SIMPLIFY-2)

- The overall population included 104 patients in the momelotinib arm vs 52 patients in the BAT arm. The ≥65-year-old population included 63 patients in the momelotinib arm vs 38 patients in the BAT arm
  - Transfusion rates at baseline and week 24 are shown in **Table 4**

Table 4. Transfusion Status at Week 24 Stratified by Baseline Transfusion Status

	Overall population		Aged ≥65 years	
n (%)	Momelotinib n=104	BAT n=52	Momelotinib n=63	BAT n=38
Baseline TD	**58 (56)**	**27 (52)**	**41 (65)**	**22 (58)**
TD at week 24	33 (57)	21 (78)	22 (54)	18 (82)
TI at week 24	19 (33)	1 (4)	14 (34)	1 (5)
TR at week 24	6 (10)	5 (19)	5 (12)	3 (14)
Baseline TI/TR	**46 (44)**	**25 (48)**	**22 (35)**	**16 (42)**
TD at week 24	19 (41)	12 (48)	10 (45)	7 (44)
TI at week 24	26 (57)	10 (40)	11 (50)	8 (50)
TR at week 24	1 (2)	3 (12)	1 (5)	1 (6)

BAT, best available therapy; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.

Projected Differences in Annual Transfusion-Related Time Burden For Patients

- Projected annual time savings in transfusion visits for momelotinib vs BAT totaled 42 hours per patient with baseline TD (**Figure 4A**), which included 16 hours of preparation/waiting, 20 hours of transfusion/recovery, and 6 hours of travel time saved
- In patients aged ≥65 years, 50 hours were projected to be saved annually for transfusion visits for momelotinib vs BAT per patients with baseline TD (**Figure 4B**), which included 20 hours of preparation/waiting, 24 hours of transfusion/recovery, and 7 hours of travel time saved (slight differences between base values and the total exist due to rounding)

Figure 4A. Annual Transfusion-Related Patient Time Burden (IBM MarketScan Commercial Database)[a]

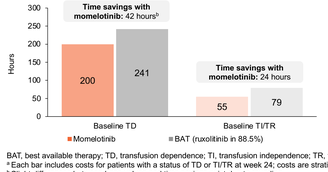


Figure 4B. Annual Transfusion-Related Time Burden in Patients Aged ≥65 Years (Medicare Database)[a]



BAT, best available therapy; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.
[a]Each bar includes costs for patients with a status of TD or TI/TR at week 24; costs are stratified by transfusion status at baseline.
[b]Slight differences between base values and time savings exist due to rounding.

Projected Differences in Total Annual Medical Costs

- In the overall population, projected average annual total medical cost savings with momelotinib vs BAT based on week 24 transfusion status were $47,484 per patient with baseline TD and $15,226 per patient with baseline TI/TR (**Figure 2A**)
- In patients aged ≥65 years, projected annual medical cost savings with momelotinib were $19,526 per patient with baseline TD (**Figure 2B**). In patients with baseline TI/TR, the projected annual savings were $42 with BAT (**Figure 2B**) due to similar week 24 TD rates for momelotinib and BAT among this subpopulation

Figure 2A. Total Annual Medical Costs (IBM MarketScan Commercial Database)[a,b]

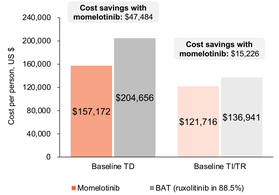
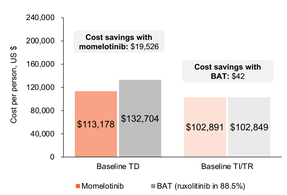


Figure 2B. Total Annual Medical Costs for Patients Aged ≥65 Years (Medicare Database)[a,b]



BAT, best available therapy; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.
[a] Costs of care were extracted from analyses of the US IBM MarketScan Commercial database or the Medicare database summarizing medical, pharmacy, and transfusion-specific costs for patients with myelofibrosis.[4]
[b] Each bar includes costs for patients with a status of TD or TI/TR at week 24; costs are stratified by transfusion status at baseline.

Projected Differences in Annual Outpatient Transfusion Visit Costs

- Fewer annual outpatient transfusion visits were recorded in the momelotinib vs BAT arms in patients who had baseline TD (13 vs 15 visits) and baseline TI/TR (4 vs 5 visits); fewer visits were associated with projected annual savings of $10,143 (baseline TD) and $5,857 (baseline TI/TR) with momelotinib (**Figure 3A**)
- In patients aged ≥65 years, projected annual outpatient transfusion visit cost savings with momelotinib vs BAT were $12,269 in patients with baseline TD and $5,427 in patients with baseline TI/TR (**Figure 3B**)

Figure 3A. Total Annual Outpatient Transfusion Visit Costs (IBM MarketScan Commercial Database)[a]

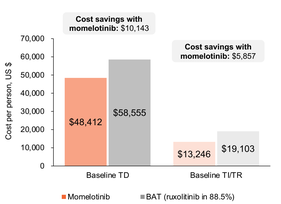
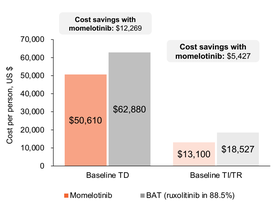


Figure 3B. Total Annual Outpatient Transfusion Visit Costs for Patients Aged ≥65 Years (Medicare Database)[a]



BAT, best available therapy; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.
[a] Each bar includes costs for patients with a status of TD or TI/TR at week 24; costs are stratified by transfusion status at baseline.

Limitations

- Due to differences in the data available across sources, definitions of TD and TI, while expected to be functionally similar, differed between SIMPLIFY-2 and IBM MarketScan and Medicare analyses
- Patient populations differed between sources: SIMPLIFY-2 included ruxolitinib-experienced patients. Estimates from the IBM MarketScan analysis are based on JAK inhibitor-naive and -experienced patients, while estimates for Medicare were available only for anemic JAK inhibitor-naive patients
- Costs for transfusion visits were not available from the Medicare analysis, therefore, cost estimates from the IBM MarketScan analysis were instead used
- Time burden for transfusions was estimated using a previous analysis in patients with transfusion-dependent β-thalassemia, as estimates for patients with MF were not available

Conclusions

- Reductions in transfusions associated with momelotinib are projected to result in savings in medical and transfusion-related costs and time burden relative to BAT in ruxolitinib-experienced patients with MF
  - These findings were consistent in patients aged ≥65 years, and in patients with TD or TI/TR status at baseline
- Future studies of real-world data are warranted to further evaluate the impact of momelotinib on the clinical, economic and humanistic burden in patients with MF
- This analysis shows that reductions in transfusions due to treatment with momelotinib can translate into meaningful cost offsets for the healthcare system and time savings for patients

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Disclosures

LM has nothing to report. **TL, SR** report employment with GSK. **MF, WL, GS, JS** report employment with Analysis Group which received consulting fees from GSK to conduct the present analysis. **BG** reports former employment with GSK at the time the present analysis was conducted, current employment with Daiichi Sankyo, and stock/stock options from GSK and Daiichi Sankyo. All authors acknowledge medical writing support for this poster, funded by GSK.

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