

# Estimating Transfusion-Related Medical Costs and Associated Time Burden in Patients With Myelofibrosis: A Comparison of Momelotinib vs Ruxolitinib Based on SIMPLIFY-1

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## Introduction

- Myelofibrosis (MF) is a myeloproliferative neoplasm characterized by splenomegaly, debilitating symptoms, and bone marrow fibrosis<sup>1,2</sup>
- Transfusion-dependent anemia is present in one-quarter of patients with MF at diagnosis and becomes more common over time<sup>1,3</sup>
- Among currently approved Janus kinase (JAK) inhibitor therapies for MF, ruxolitinib (RUX) and fedratinib may induce or worsen anemia, and management strategies for patients with anemia have limited effectiveness<sup>3</sup>
- 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 for patients with TD up to 9 times higher than costs for those without TD<sup>3,4</sup>
- Momelotinib is a JAK1/JAK2/activin A receptor type 1 (ACVR1) inhibitor recently approved for the treatment of patients with MF and anemia, and has demonstrated improvement in anemia and TD, in addition to symptom and spleen responses, across 3 phase 3 trials, including SIMPLIFY-1 (NCT01969838)<sup>5-8</sup>
- SIMPLIFY-1 was a randomized, phase 3 trial that evaluated momelotinib vs RUX in JAK inhibitor-naïve patients with MF. Fewer patients treated with momelotinib were transfusion dependent at week 24 (30.2% vs 40.1%) and a higher proportion became transfusion independent (66.5% vs 49.3%) than those who received RUX<sup>9</sup>

## Objective

- To estimate projected differences between momelotinib and RUX in medical costs, transfusion-related costs, and patient time burden among JAK inhibitor-naïve patients with MF

## Methods

- Transfusion-related cost and time differences were calculated based on week 24 transfusion data from SIMPLIFY-1 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 in medical care and outpatient transfusion visits for patients with MF in SIMPLIFY-1 were calculated for (1) the overall study population, (2) an anemia subgroup (Hb <10 g/dL at baseline), and (3) patients aged ≥65 years. Cost estimates from the US IBM MarketScan Commercial database<sup>4</sup> were used for the overall population and the anemia subgroup; cost estimates from the Medicare fee-for-service database were used for patients aged ≥65 years (Table 2)
- Projected time savings were based on transfusion rates calculated from SIMPLIFY-1, and time burden estimates were based on literature in patients with transfusion-dependent β-thalassemia (Table 3)<sup>9</sup>
- The mean number of visits for red blood cell (RBC) transfusions was reported in the SIMPLIFY-1 Clinical Study Report for the overall population. For patients with anemia at baseline and patients aged ≥65 years, this was calculated using individual patient-level data. 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

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

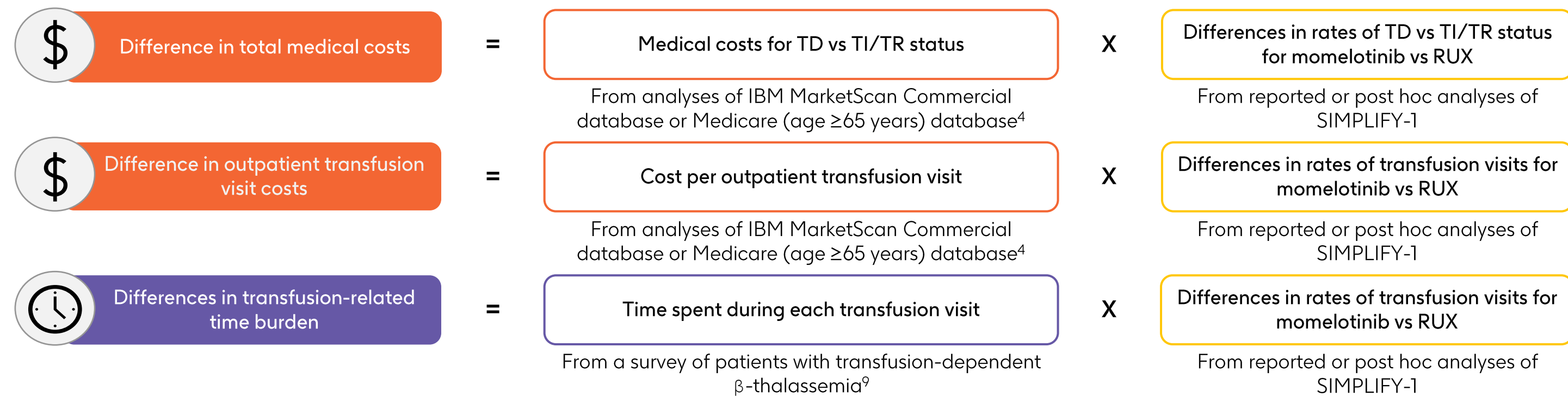


Table 1. Criteria for Transfusion Status<sup>a</sup>

	SIMPLIFY-1 <sup>b</sup>	IBM MarketScan Commercial analysis <sup>c</sup>	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

<sup>a</sup> Definitions were assumed as being functionally similar, as each study defines TI status as zero RBC transfusions and TD status as equal to a 0.5-unit transfusion per week.

Table 2. Estimated Costs of Care<sup>4</sup>

Outcome	TI/TR <sup>a</sup>	TD <sup>b</sup>	All patients
IBM MarketScan Commercial database analysis <sup>b</sup>			
Total per-person per-year medical cost, US \$	27,789	255,190	–
Outpatient transfusion visit cost, US \$ <sup>c</sup>	–	–	3790
Medicare database analysis			
Total per-person per-year medical cost, US \$	TI: 69,961 TR: 133,561	136,047	98,223
Outpatient transfusion visit cost, US \$ <sup>c</sup>	–	–	3790

<sup>a</sup> As defined in previous studies of IBM MarketScan Commercial or Medicare databases (Table 1). <sup>b</sup> Estimates were based on JAK inhibitor-naïve and JAK inhibitor-experienced patients. <sup>c</sup> It was assumed that the mean cost per transfusion was equivalent to the mean cost for an outpatient transfusion claim. <sup>d</sup> Estimates were available for JAK inhibitor-naïve 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<sup>9</sup>

Outcome	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

## Results

### Transfusion Status at Baseline and Week 24 (SIMPLIFY-1)

- The overall population included 215 patients in the momelotinib arm and 217 patients in the RUX arm. The anemia subgroup (Hb <10 g/dL at baseline) included 86 patients in the momelotinib arm and 94 patients in the RUX arm. The ≥65-year-old population included 125 patients in the momelotinib arm and 122 patients in the RUX 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		Anemia subgroup (Hb <10 g/dL at baseline)		Aged ≥65 years	
n (%)	Momelotinib (n=215)	RUX (n=217)	Momelotinib (n=86)	RUX (n=94)	Momelotinib (n=125)	RUX (n=122)
Baseline TD	53 (25)	52 (24)	49 (57)	43 (46)	40 (32)	31 (25)
TD at week 24	33 (62)	37 (71)	29 (59)	31 (72)	26 (65)	23 (74)
TI at week 24	16 (30)	9 (17)	16 (33)	8 (19)	12 (30)	4 (13)
TR at week 24	4 (8)	6 (12)	4 (8)	4 (9)	2 (5)	4 (13)
Baseline TI/TR	162 (75)	165 (76)	37 (43)	51 (54)	85 (68)	91 (75)
TD at week 24	32 (20)	50 (30)	12 (32)	27 (53)	21 (25)	33 (36)
TI at week 24	127 (78)	98 (59)	24 (65)	17 (33)	63 (74)	43 (47)
TR at week 24	3 (2)	17 (10)	1 (3)	7 (14)	1 (1)	15 (16)

### Projected Differences in Total Annual Medical Costs

- In the overall population, projected average annual total medical cost savings with momelotinib vs RUX based on week 24 transfusion status were \$20,215 per patient with baseline TD and \$23,991 per patient with baseline TI/TR (Figure 2A)
- In the anemia subgroup, projected average annual medical cost savings with momelotinib were \$29,356 per patient with baseline TD and \$46,637 per patient with baseline TI/TR (Figure 2B)
- In patients aged ≥65 years, projected annual medical cost savings with momelotinib were \$11,102 per patient with baseline TD and \$17,373 per patient with baseline TI/TR (Figure 2C)

Figure 2A. Total Annual Medical Costs (IBM MarketScan Commercial Database)<sup>a,b</sup>

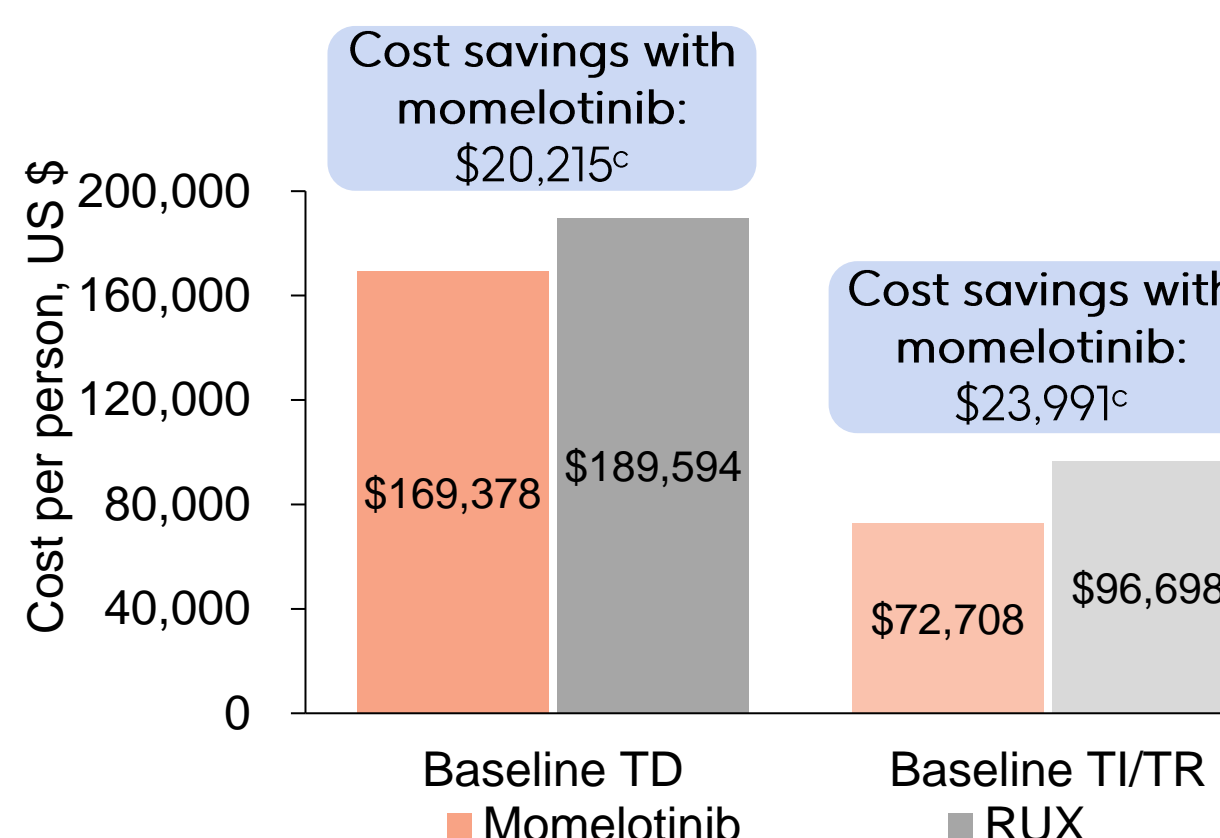


Figure 2B. Total Annual Medical Costs for Patients with Anemia (IBM MarketScan Commercial Database)<sup>a,b</sup>

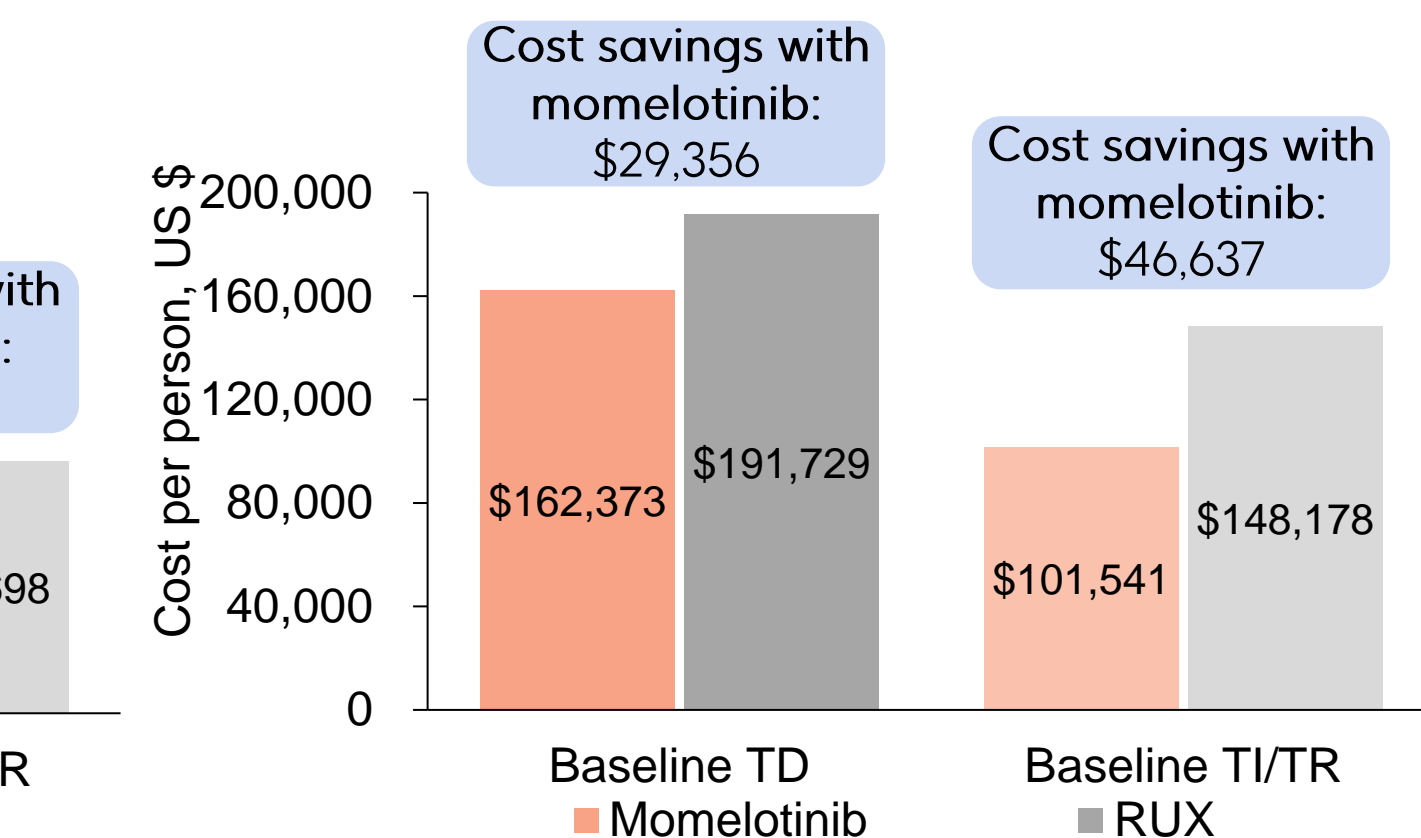
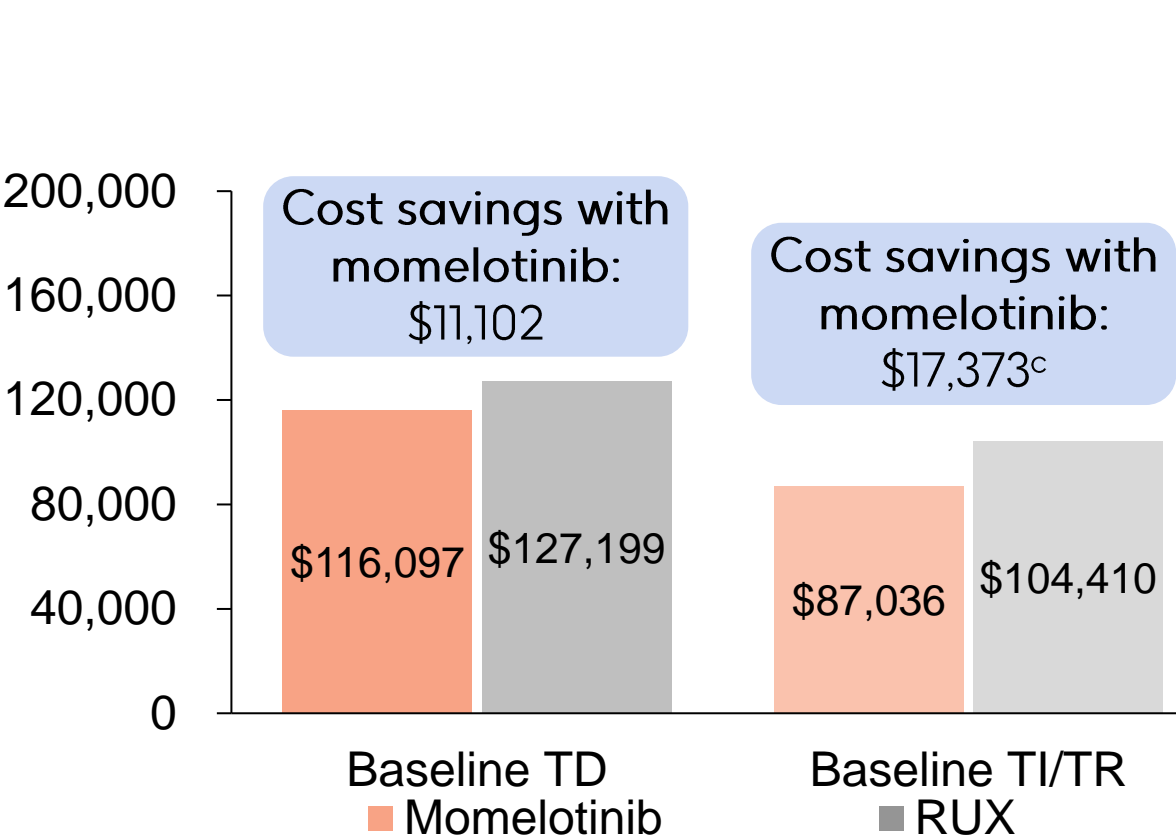


Figure 2C. Total Annual Medical Costs for Patients Aged ≥ 65 Years (Medicare Database)<sup>a,b</sup>



<sup>a</sup> 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 MF.<sup>10</sup> <sup>b</sup> 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. <sup>c</sup> Slight differences between base values and cost savings exist due to rounding.

### Projected Differences in Annual Outpatient Transfusion Visit Costs

- Fewer annual outpatient transfusion visits were recorded in the momelotinib arm than in the RUX arm in patients who had baseline TD (9.39 vs 16.17 visits) and baseline TI/TR (0.63 vs 3.82 visits); fewer visits were associated with projected annual savings of \$25,704 (baseline TD) and \$12,083 (baseline TI/TR) with momelotinib (Figure 3A)
- In the anemia subgroup, projected annual outpatient transfusion visit cost savings with momelotinib was \$29,767 in patients with baseline TD and \$21,133 in patients with baseline TI/TR (Figure 3B)
- In patients aged ≥65 years, projected annual outpatient transfusion visit cost savings with momelotinib was \$28,826 in patients with baseline TD and \$18,255 in patients with baseline TI/TR (Figure 3C)

Figure 3A. Total Annual Outpatient Transfusion Visit Costs (IBM MarketScan Commercial Database)<sup>a</sup>

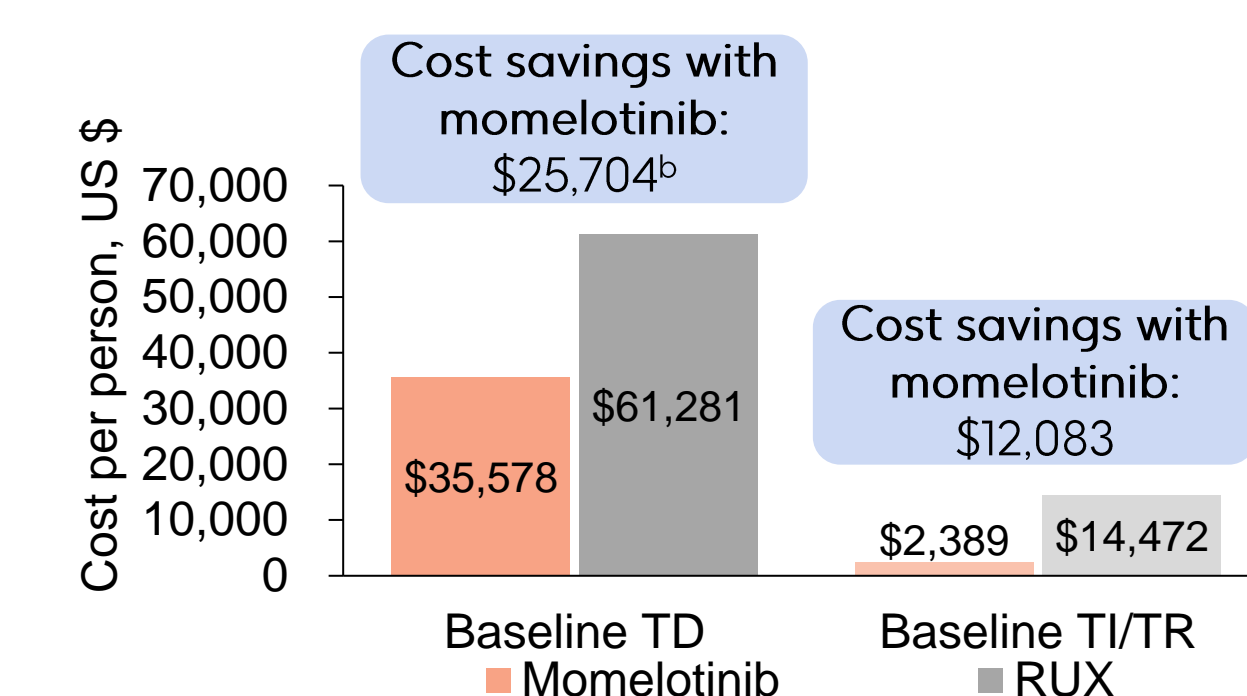


Figure 3B. Total Annual Outpatient Transfusion Visit Costs for Patients with Anemia (IBM MarketScan Commercial Database)<sup>a</sup>

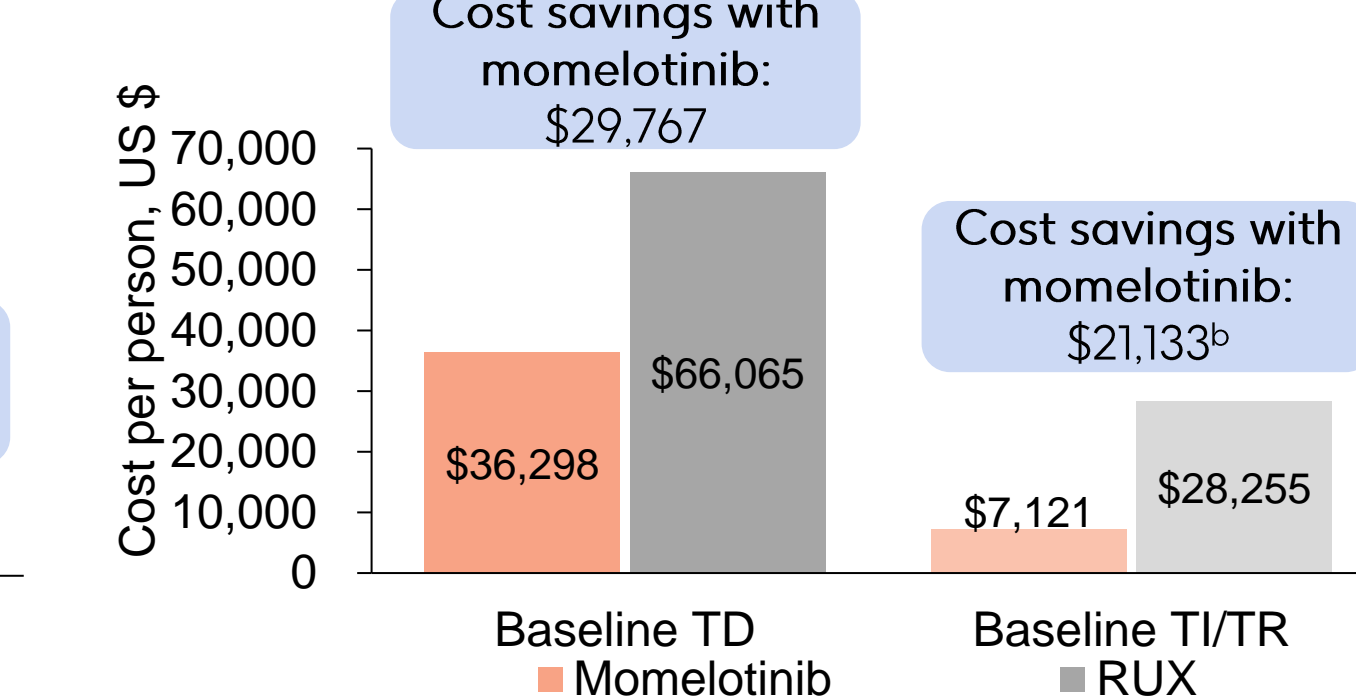
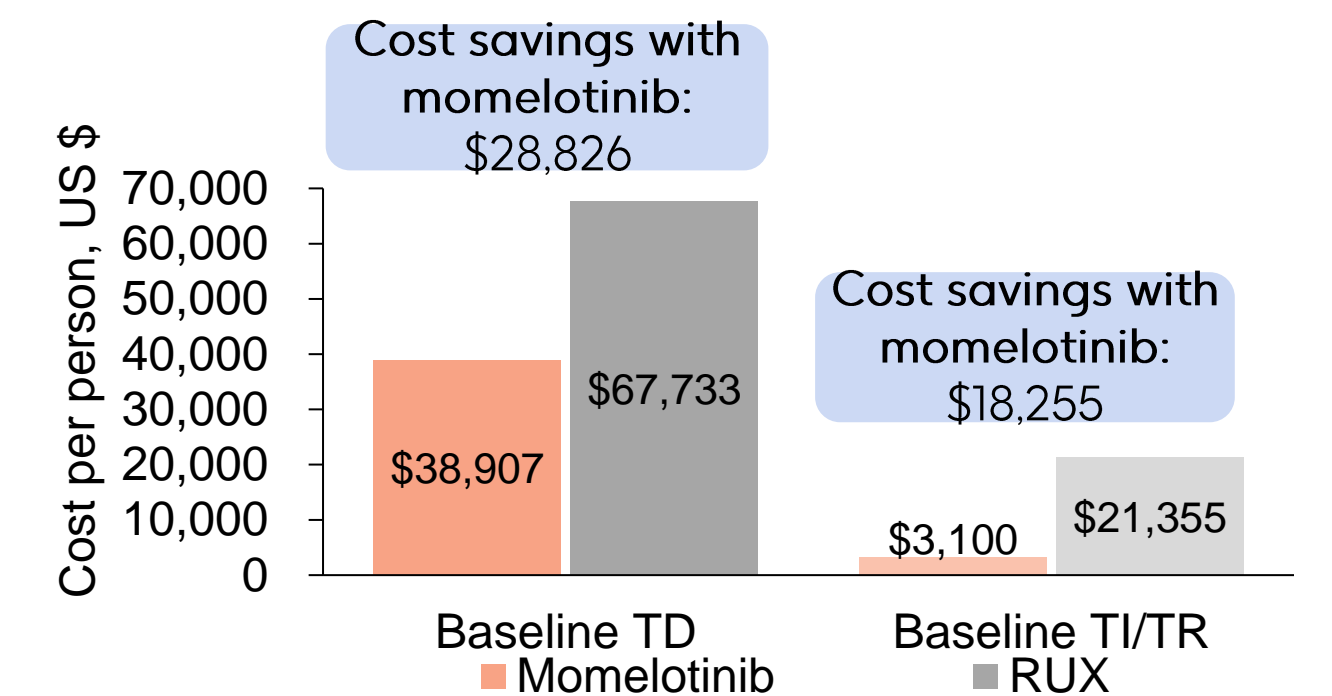


Figure 3C. Total Annual Outpatient Transfusion Visit Costs for Patients Aged ≥ 65 Years (Medicare Database)<sup>a</sup>



<sup>a</sup> 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. <sup>b</sup> Slight differences between base values and cost savings exist due to rounding.

### Projected Differences in Annual Transfusion-Related Time Burden for Patients

- Projected annual patient time savings in transfusion visits for momelotinib vs RUX totaled 106 hours per patient with baseline TD (Figure 4A), which included 42 hours of preparation/waiting, 50 hours of transfusion/recovery, and 15 hours of travel time saved
- In the anemia subgroup, 123 hours were projected to be saved annually for transfusion visits for momelotinib vs RUX per patient with baseline TD (Figure 4B)
- In patients aged ≥65 years, 119 hours were projected to be saved annually for transfusion visits for momelotinib vs RUX per patients with baseline TD (Figure 4C), which included 47 hours of preparation/waiting, 56 hours of transfusion/recovery, and 16 hours of travel time saved

Figure 4A. Annual Transfusion-Related Patient Time Burden (IBM MarketScan Commercial Database)<sup>a</sup>

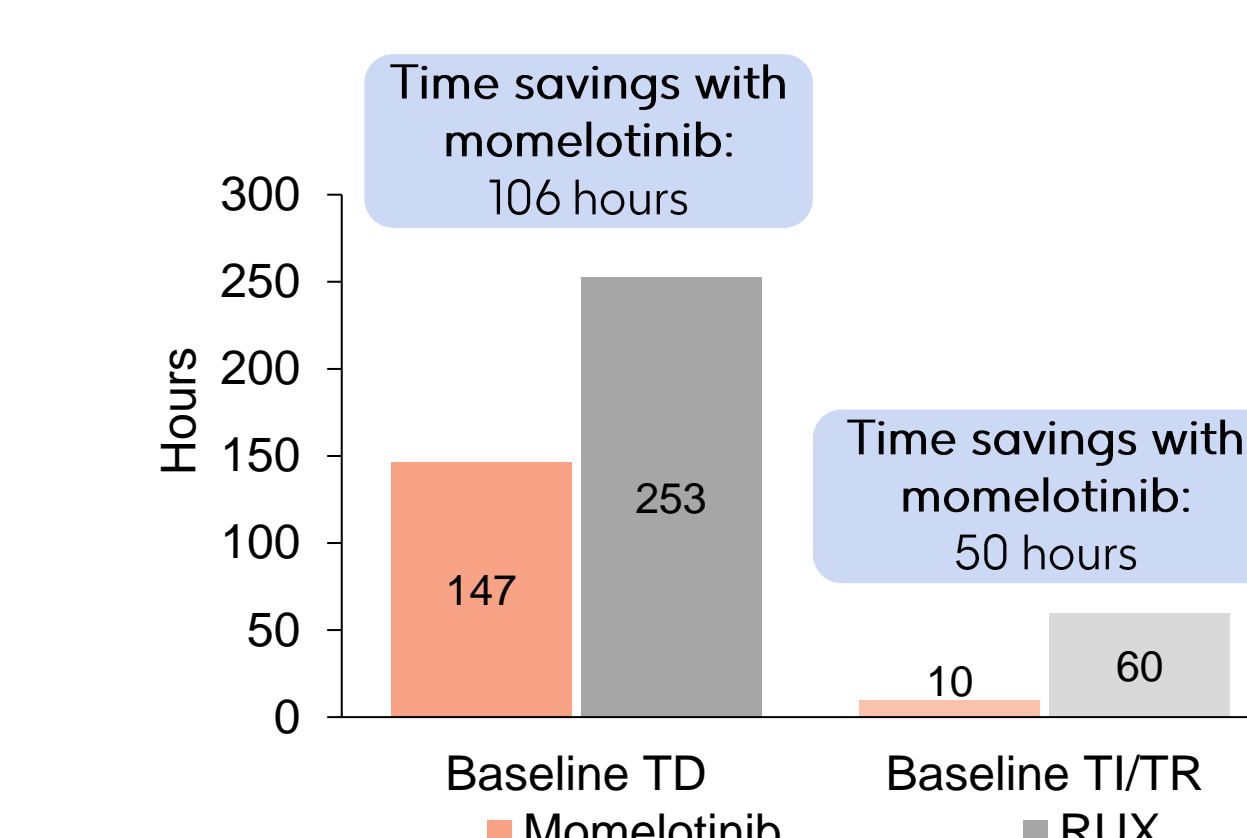


Figure 4B. Annual Transfusion-Related Time Burden for Patients with Anemia (IBM MarketScan Commercial Database)<sup>a</sup>

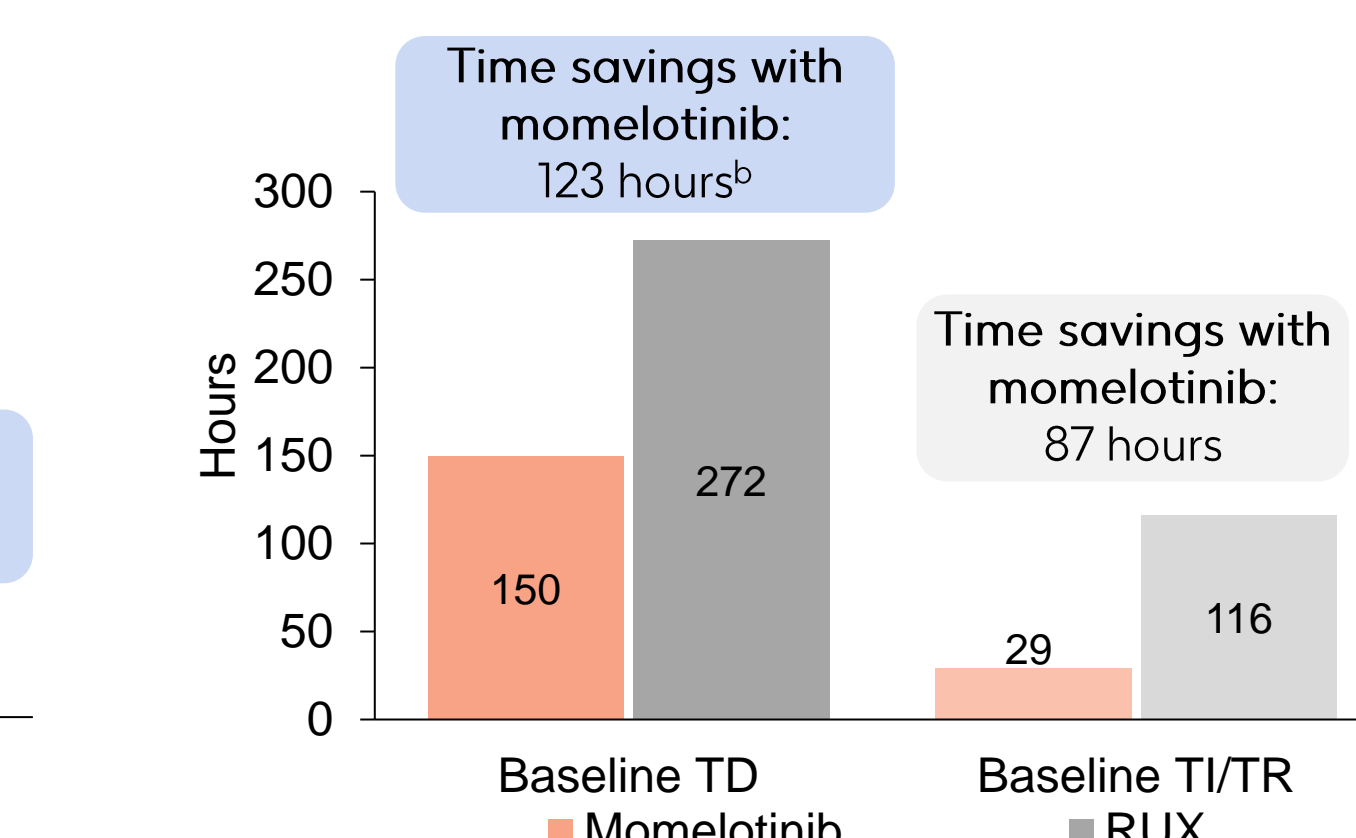
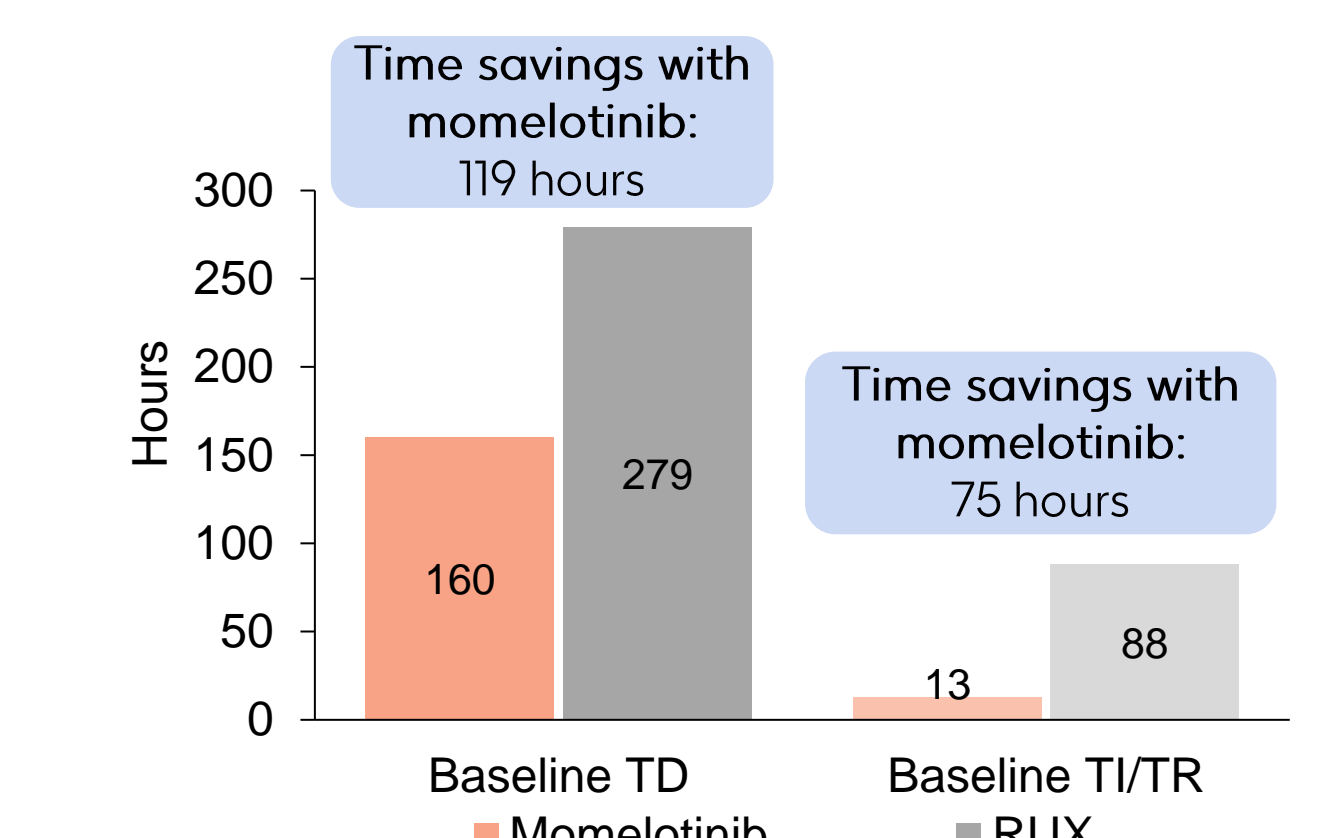


Figure 4C. Annual Transfusion-Related Time Burden for Patients Aged ≥ 65 Years (Medicare Database)<sup>a</sup>



<sup>a</sup> Each bar includes time burden for patients with a status of TD or TI/TR at week 24; time burdens are stratified by transfusion status at baseline. <sup>b</sup> Slight differences between base values and cost savings exist due to rounding.

## Abbreviations

ACVR1, activin A receptor type 1; Hb, hemoglobin; JAK, Janus kinase; MF, myelofibrosis; RBC, red blood cell; RUX, ruxolitinib; TD, transfusion dependence; TI, transfusion independence; TR, transfusion requiring.

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