Supplementary Online Content

Mascarenhas J, Hoffman R, Talpaz M, et al. Pacritinib vs best available therapy, including ruxolitinib, in patients with myelofibrosis: a randomized clinical trial. *JAMA Oncol*. Published online March 8, 2018. doi:10.1001/jamaoncol.2017.5818

This supplementary material has been provided by the authors to give readers additional information about their work.

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eMethods

Additional exclusion criteria:

- Prior splenectomy or hematopoietic cell transplantation, or plans to undergo either were not allowed
- Full list of excluded significant cardiac abnormalities:
  - Clinically symptomatic and uncontrolled cardiovascular disease
  - History of any of the following within 6 months prior to randomization: myocardial infarction, severe/unstable angina, or symptomatic congestive heart failure
  - New York Heart Association Class III-IV congestive heart failure
  - Ongoing National Cancer Institute Common Terminology Criteria for Adverse Events grade ≥3 cardiac dysrhythmias
    - Patients with grade 2 cardiac arrhythmias may have been considered for inclusion with the approval of the medical monitor if the arrhythmias were stable, asymptomatic, and unlikely to affect patient safety
  - Corrected QT prolongation >450 ms, or other factors that increase risk for QT prolongation (eg heart failure, hypokalemia [<3.0 mEq/L that is persistent and refractory to correction], or family history of long QT interval syndrome)

Details on Randomization Stratification

- Geographic regions: United States, Canada, Europe, Rest of the World
- Risk categories: intermediate-1, intermediate-2, high
- Rebound platelet count: $\leq 100\times10^9$/L vs. $>100\times10^9$/L
  - To be included in the $>100\times10^9$/L group, patients met both of the following criteria:
    1) rebound platelet count $>100\times10^9$/L *and*
    2) >50% increase above their first qualifying platelet value after consent.

Note: the first qualifying platelet value after informed consent and the most recent platelet count obtained prior to randomization was the basis for determining platelet rebound stratification. For patients who received any platelet transfusions, a pre-transfusion platelet count was obtained within 8 hours prior to transfusion, and that platelet count was used for stratification. If >1 such nadir count was obtained prior to randomization, the count obtained closest to randomization was used for stratification. If patients received frequent platelet transfusions and platelet counts were obtained, clinical judgment was used to identify the most appropriate nadir platelet count obtained prior to the last transfusion before randomization.

Definitions of Progressive Disease

- Increase in spleen volume ≥25% from baseline by CT/MRI, or
- Investigator decision to pursue splenic irradiation or splenectomy, or
- Leukemic transformation

Standardized MedDRA Queries (SMQs)

Cardiac events defined by SMQ are the preferred terms in the SMQs of Cardiac Arrhythmias, Cardiac Failure, Ischemic Heart Disease, and Embolic and Thrombotic Events. The events within these SMQs noted in this study were: cardiac failure (edema peripheral, cardiac failure, cardiac failure congestive, pulmonary edema, ejection fraction decreased, cardiac failure acute, left ventricular dysfunction, cardiorenal syndrome, edema and right ventricular failure); cardiac arrhythmias (cardiac arrhythmia terms SMQ, conduction defects SMQ [electrocardiogram QT prolonged], disorders of sinus node function SMQ, tachyarrhythmias SMQ, supraventricular tachyarrhythmias SMQ [atrial fibrillation, arrhythmia supraventricular, sinus tachycardia, atrial flutter, supraventricular tachycardia], arrhythmia related investigations, signs and symptoms SMQ [palpitations, cardiac arrest, syncope, tachycardia, sudden death]); embolic and thrombotic events (embolic and thrombotic events, venous SMQ [pulmonary embolism, intracranial venous sinus thrombosis, mesenteric vein thrombosis, retinal vein occlusion, deep vein thrombosis], embolic and thrombotic events, vessel type unspecified and mixed arterial and venous SMQ [splenic infarction, cerebrovascular accident], embolic and thrombotic events, arterial SMQ [myocardial infarction, transient ischemic attack]); ischemic heart disease (other ischemic heart disease SMQ [angina pectoris, ischemic cardiomyopathy], myocardial infarction SMQ [myocardial infarction]).

Bleeding events defined by SMQ are the preferred terms in the Hemorrhages SMQ. The events within these SMQs noted in this study were: epistaxis, contusion, conjunctival hemorrhage, hematoma, petechiae, purpura, ecchymosis, rectal hemorrhage, gingival bleeding, post procedural hemorrhage, retinal hemorrhage, gastritis hemorrhagic, gastrointestinal hemorrhage, hemoptysis, melena, mouth hemorrhage, muscle hemorrhage, esophageal varices hemorrhage, periorbital contusion, subdural hematoma, blood blister, cerebral hemorrhage, duodenal ulcer hemorrhage, gastric varices hemorrhage, hematochezia, hematuria, hemorrhage intracranial, hemorrhoidal hemorrhage, meningorrhagia, esophageal hemorrhage, post procedural contusion, post procedural hematoma, shock hemorrhagic, skin hemorrhage, subcutaneous hematoma, tongue hematoma, tooth socket hemorrhage, urethral hemorrhage, vaginal hemorrhage, vessel puncture site bruise, eye contusion, increased tendency to bruise, infusion site bruising, small intestinal hemorrhage, splenic hematoma, traumatic hematoma; laboratory terms include prothrombin time prolonged, hemoglobin decreased, activated partial thromboplastin time prolonged and international normalized ratio increased.

eTable 1. Best Available Therapy Received in ≥1 Patient

	BAT (n=98), n (%)
Ruxolitinib[a]	44 (45)
Watch and wait only	19 (19)
Hydroxyurea (hydroxycarbamide)	19 (19)
Prednisone/prednisolone	13 (13)
Danazol	5 (5)
Thalidomide	3 (3)
Decitabine	2 (2)
Interferon-alpha	2 (2)

[a]17 (39%) had baseline platelet counts $<50\times10^9$/L and would not have been candidates for ruxolitinib by approved indication (or PERSIST-2 study protocol).
Abbreviation: BAT, best available therapy.

eTable 2. Patient Demographics and Disease Characteristics (Intention-to-Treat Efficacy Population)

	Pacritinib 400 mg once daily (n=75)	Pacritinib 200 mg twice daily (n=74)	BAT (n=72)
Median age, years (range)	69 (39-85)	67 (39-85)	69 (32-83)
≥65 years, n (%)	53 (71)	46 (62)	51 (71)
Male, n (%)	38 (51)	48 (65)	39 (54)
ECOG performance status, n (%)			
0-1	57 (76)	65 (88)	54 (75)
2-3	17 (23)	8 (11)	15 (21)
Missing	1 (1)	1 (1)	3 (4)
Myelofibrosis diagnosis, n (%)			
Primary	46 (61)	55 (74)	43 (60)
Post-polycythemia vera	16 (21)	14 (19)	16 (22)
Post-essential thrombocythemia	13 (17)	5 (7)	13 (18)
DIPSS risk category, n (%)			
Intermediate-1	13 (17)	14 (19)	13 (18)
Intermediate-2	40 (53)	38 (51)	37 (51)
High	22 (29)	22 (30)	22 (31)
$JAK2^{V617F}$ positive, n (%)	60 (80)	59 (80)	51 (71)
Median palpable spleen length,[a] cm (range)	13 (3-33)	15 (5-32)	13 (2-34)
Bone marrow biopsy completed, n	73	74	72
Reticulin and collagen fibrosis staging, n (%)	4 (5)	1 (1)	4 (6)
MF-0	1 (1)	6 (8)	6 (8)
MF-1	23 (32)	19 (26)	21 (29)
MF-2	38 (52)	46 (62)	36 (50)
MF-3	7 (10)	2 (3)	5 (7)
Missing			
Platelet count $<50\times10^9$/L, n (%)	38 (51)	31 (42)	32 (44)
Hemoglobin <10 g/dL, n (%)	45 (60)	44 (59)	41 (57)
Peripheral blasts category, n (%)			
0-<5%	62 (83)	61 (82)	60 (83)
≥5%	9 (12)	7 (9)	7 (10)
Missing	4 (5)	6 (8)	5 (7)
WBC count $>25 \times 10^9$/L, n (%)	15 (20)	17 (23)	14 (19)
RBC transfusion dependence,[b] n (%)			
Dependent	17 (23)	14 (19)	14 (19)
Independent	38 (51)	37 (50)	37 (51)
Indeterminate	20 (27)	22 (30)	21 (29)
Missing	0	1 (1)	0
Prior therapies, n (%)			
0	21 (28)	18 (24)	17 (24)
1	18 (24)	25 (34)	21 (29)
2	12 (16)	16 (22)	16 (22)
3	12 (16)	5 (7)	9 (13)
≥3	12 (16)	10 (14)	9 (13)
Prior JAK2 inhibitors, n (%)	33 (44)	33 (45)	34 (47)
Prior ruxolitinib	31 (41)	31 (42)	33 (46)

Abbreviaton: DIPSS, Dynamic International Prognostic Scoring System; ECOG, Eastern Cooperative Oncology Group; RBC, red blood cell; WBC, white blood cell.
[a]By physical examination. [b]Defined according to Gale criteria.

eTable 3. Treatment Outcomes and Treatment-Emergent Adverse Events (Safety Population)

	Pacritinib 400 mg once daily (n=104)	Pacritinib 200 mg twice daily (n=106)	BAT (n=98)
Median exposure, weeks (range)	23 (1-82)	25 (1-84)	21 (1-56)
Discontinuation for any reason, n (%)	104 (100)	106 (100)	98 (100)
Other,[a] n (%)	65 (63)	79 (75)	33 (34)
Adverse events, n (%)	15 (14)	10 (9)	4 (4)
Withdrawal by patient, n (%)	9 (9)	5 (5)	4 (4)
Physician decision,[b] n (%)	5 (5)	3 (3)	41 (42)
Progressive disease, n (%)	5 (5)	7 (7)	11 (11)
Death, n (%)	5 (5)	2 (2)	5 (5)
Dose modifications due to adverse events, n (%)			
Dose interruptions	39 (38)	29 (27)	10 (10)
Dose reductions	21 (20)	13 (12)	7 (7)
Discontinuations	20 (19)	16 (15)	12 (12)
Any adverse event, n (%)	104 (100)	100 (94)	87 (89)
Any grade adverse event in ≥15% in any arm, n (%)			
Diarrhea	70 (67)	51 (48)	15 (15)
Nausea	39 (38)	34 (32)	11 (11)
Thrombocytopenia	34 (33)	36 (34)	23 (23)
Anemia	29 (28)	25 (24)	15 (15)
Vomiting	22 (21)	20 (19)	5 (5)
Fatigue	18 (17)	18 (17)	16 (16)
Peripheral edema	14 (13)	21 (20)	15 (15)
Dizziness	15 (14)	16 (15)	5 (5)
Abdominal pain	20 (19)	10 (9)	19 (19)
Pyrexia	11 (11)	16 (15)	3 (3)
Grade 3/4 adverse events in ≥5% in any arm, n (%)			
Any grade 3/4 adverse events	79 (76)	74 (70)	48 (49)
Thrombocytopenia	32 (31)	34 (32)	18 (18)
Anemia	28 (27)	23 (22)	14 (14)
Neutropenia	9 (9)	7 (7)	5 (5)
Pneumonia	4 (4)	7 (7)	3 (3)
Fatigue	7 (7)	3 (3)	5 (5)
Diarrhea	5 (5)	4 (4)	0
Epistaxis	2 (2)	5 (5)	1 (1)
Serious adverse events in ≥5% in any arm, n (%)			
Any serious adverse event	48 (46)	50 (47)	30 (31)
Anemia	5 (5)	8 (8)	3 (3)
Thrombocytopenia	2 (2)	6 (6)	2 (2)
Pneumonia	5 (5)	6 (6)	4 (4)
Renal failure, acute	5 (5)	2 (2)	2 (2)
Additional serious adverse events of interest, n (%)			
Congestive heart failure	1 (1)	4 (4)	2 (2)
Atrial fibrillation	3 (3)	0	3 (3)
Cardiac arrest	2 (2)	0	0
Epistaxis	2 (2)	2 (2)	1 (1)
Subdural hematoma	2 (2)	0	0

[a]Majority of "other" were due to placement of the clinical hold.
[b]Majority of "physician decision" in the BAT arm were due to patient crossover to pacritinib treatment.
Abbreviation: BAT, best available therapy.

eTable 4. Pacritinib Exposure.

	Regimen	n	Geometric Mean	Ratio	90% CI
$Cmin_{ss}$	200 mg twice daily	83	6783	1.12	0.95-1.31
	400 mg once daily	76	6076		
$Cmax_{ss}$	200 mg twice daily	36	8290	0.93	0.77-1.12
	400 mg once daily	35	8930		

Abbreviations: CI, confidence interval; $Cmax_{ss}$, steady-state maximum concentration; $Cmin_{ss}$, steady-state minimum serum concentration.

eTable 5. Time to Onset of First Diarrhea (Safety Population).

	Pacritinib 400 mg once daily (n=104)	Pacritinib 200 mg twice daily (n=106)	BAT (n=98)
Patients with any diarrhea event, n (%)	70 (67%)	51 (48%)	15 (15%)
Mean time to onset (SD), weeks	2.49 (7.005)	3.27 (4.486)	9.47 (11.197)
Median time to onset (IQR) [range], weeks	0.29 (0.14-1.00) [0.14-48.14]	1.14 (0.43-4.29) [0.14-21.29]	3.86 (0.71-21.86) [0.14-27.29]

Abbreviations: BAT, best available therapy; IQR, inter quartile range; SD, standard deviation.

eTable 6: Discontinuations Due to Adverse Events (Safety Population)

Discontinuations in ≥2 Patient on Study, n (%)	Pacritinib 400 mg once daily (n=104)	Pacritinib 200 mg twice daily (n=106)	BAT (n=98)
Any	20 (19)	16 (15)	12 (12)
Thrombocytopenia	4 (4)	2 (2)	2 (2)
Anemia	2 (2)	3 (3)	0
Neutropenia	2 (2)	1 (1)	0
Disease progression	2 (2)	1 (1)	1 (1)
Abdominal pain	1 (1)	0	1 (1)
Diarrhea	2 (2)	0	0
Decreased appetite	1 (1)	0	1 (1)
Hyponatremia	2 (2)	0	0
Epistaxis	0	1 (1)	1 (1)

Abbreviation: BAT, best available therapy.

eTable 7. Summary of On-Study Deaths (Intention-To-Treat Population).

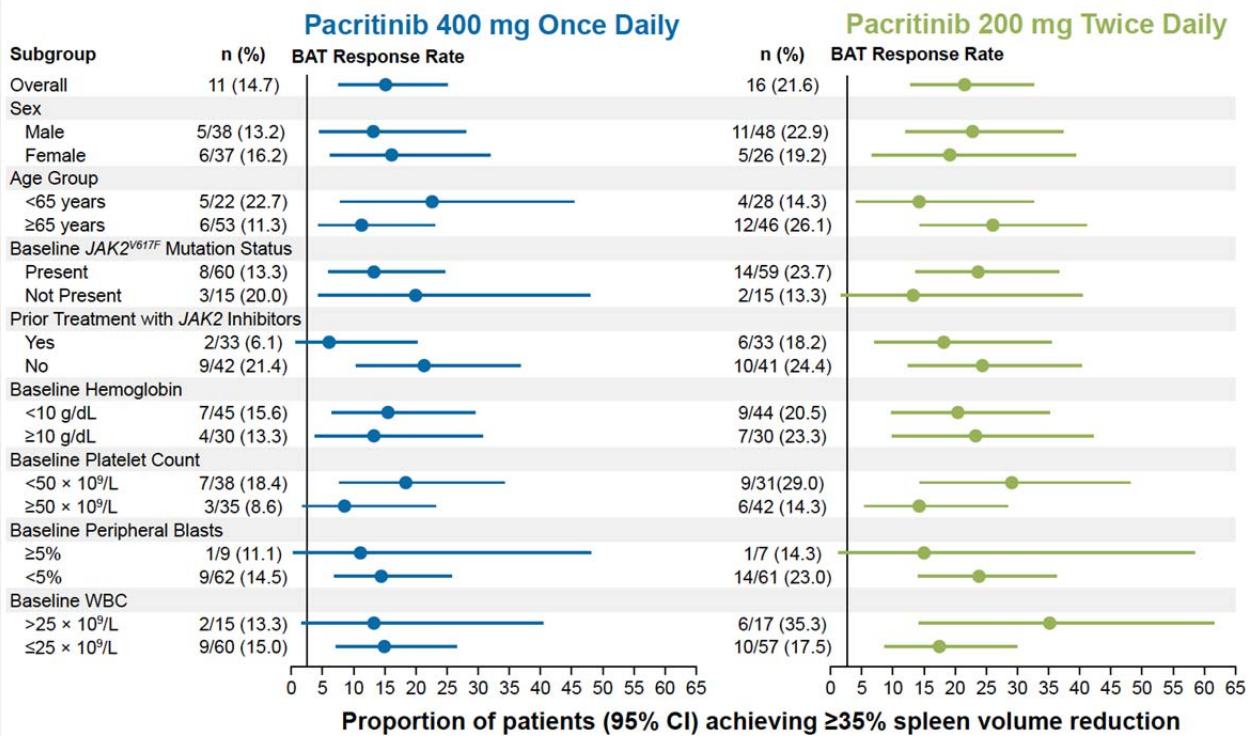
	Pacritinib 400 mg once daily (n=104)	Pacritinib 200 mg twice daily (n=107)	BAT[a] (n=100)
All on-study deaths,[b] n (%)	14 (14)	6 (6)	9 (9)
Due to adverse events, n (%)	11 (11)	3 (3)	5 (5)
Cardiac events	3 (3)	0	1 (1)
Bleeding events	1 (1)	2 (2)	1 (1)
Due to progressive disease, n (%)	3 (3)	3 (3)	4 (4)

[a]2 of 9 BAT patients who died did so after crossover to pacritinib.
[b]Defined as deaths occurring while patients are on treatment or within 30 days of treatment discontinuation.
Abbrevaition: BAT, best available therapy.

eTable 8: Cardiac and Bleeding Events (Safety Population).

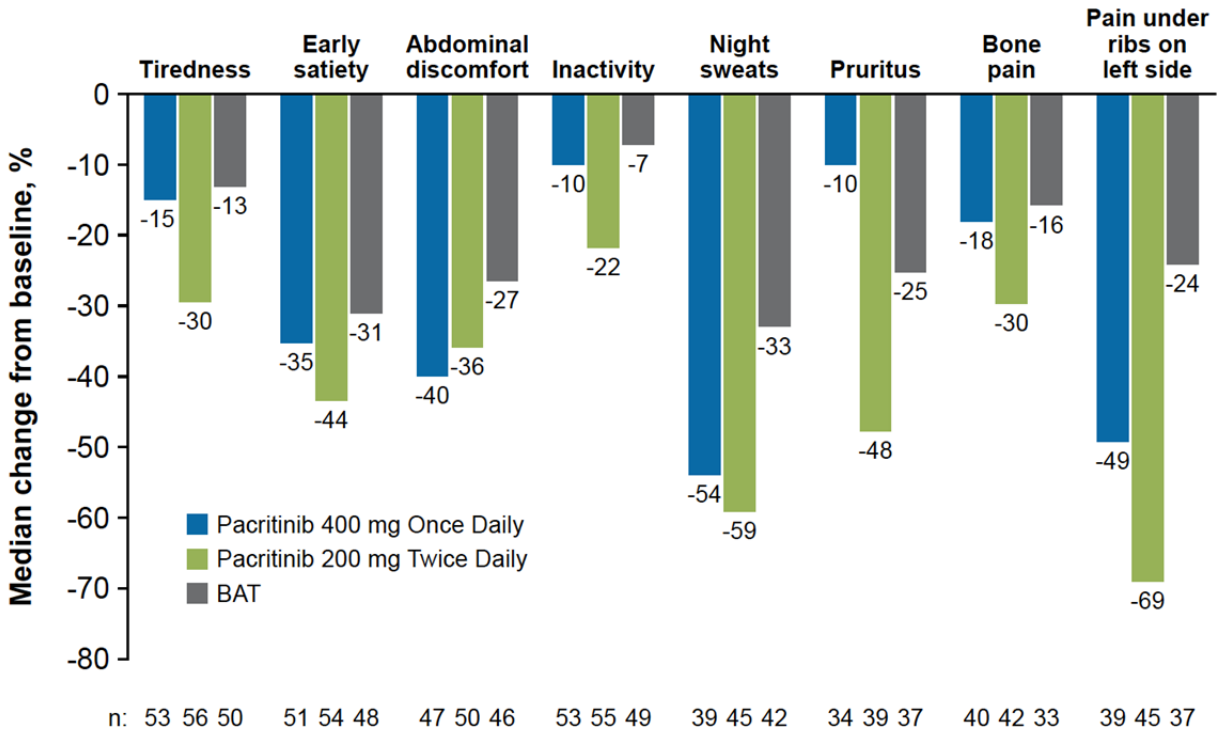
Any-Grade Events in ≥3 Patients or Grade 3/4 Events in ≥2 Patient in Any Arm	Pacritinib 400 mg once daily (n=104)		Pacritinib 200 mg twice daily (n=106)		BAT (n=98)	
	Any Gr	Gr 3/4	Any Gr	Gr 3/4	Any Gr	Gr 3/4
Cardiac events (standardized MedDRA query), n (%)						
Any	33 (32)	13 (13)	34 (32)	7 (7)	27 (28)	9 (9)
Peripheral edema	14 (13)	0	21 (20)	1 (1)	15 (15)	0
ECG QT prolonged	6 (6)	1 (1)	2 (2)	0	2 (2)	1 (1)
Atrial fibrillation	5 (5)	3 (3)	0	0	4 (4)	2 (2)
Cardiac failure	2 (2)	2 (2)	4 (4)	3 (3)	3 (3)	2 (2)
Ejection fraction decreased	2 (2)	2 (2)	0	0	0	0
Palpitations	0	0	3 (3)	0	1 (1)	0
Bleeding events (standardized MedDRA query), n (%)						
Any	37 (36)	7 (7)	45 (42)	15 (14)	40 (41)	7 (7)
Epistaxis	11 (11)	2 (2)	13 (12)	5 (5)	13 (13)	1 (1)
Contusion	7 (7)	0	10 (9)	0	8 (8)	0
Petechiae	4 (4)	0	4 (4)	0	4 (4)	1 (1)
Ecchymosis	4 (4)	0	2 (2)	0	4 (4)	0
Hematoma	3 (3)	1 (1)	6 (6)	0	2 (2)	0
Conjunctival hemorrhage	3 (3)	0	6 (6)	2 (2)	4 (4)	0
Subdural hematoma	2 (2)	2 (2)	0	0	0	0
Post-procedural hemorrhage	1 (1)	0	2 (2)	2 (2)	1 (1)	1 (1)
Increased tendency to bruise	0	0	0	0	4 (4)	0
Retinal hemorrhage	0	0	3 (3)	1 (1)	1 (1)	0
Purpura	0	0	7 (7)	0	0	0
Hemorrhagic gastritis	0	0	2 (2)	2 (2)	0	0
Mouth hemorrhage	0	0	2 (2)	2 (2)	1 (1)	0

Abbreviations: BAT, best available therapy; ECG, electrocardiogram; Gr, grade; MedDRA, Medical Dictionary for Regulatory Activities.

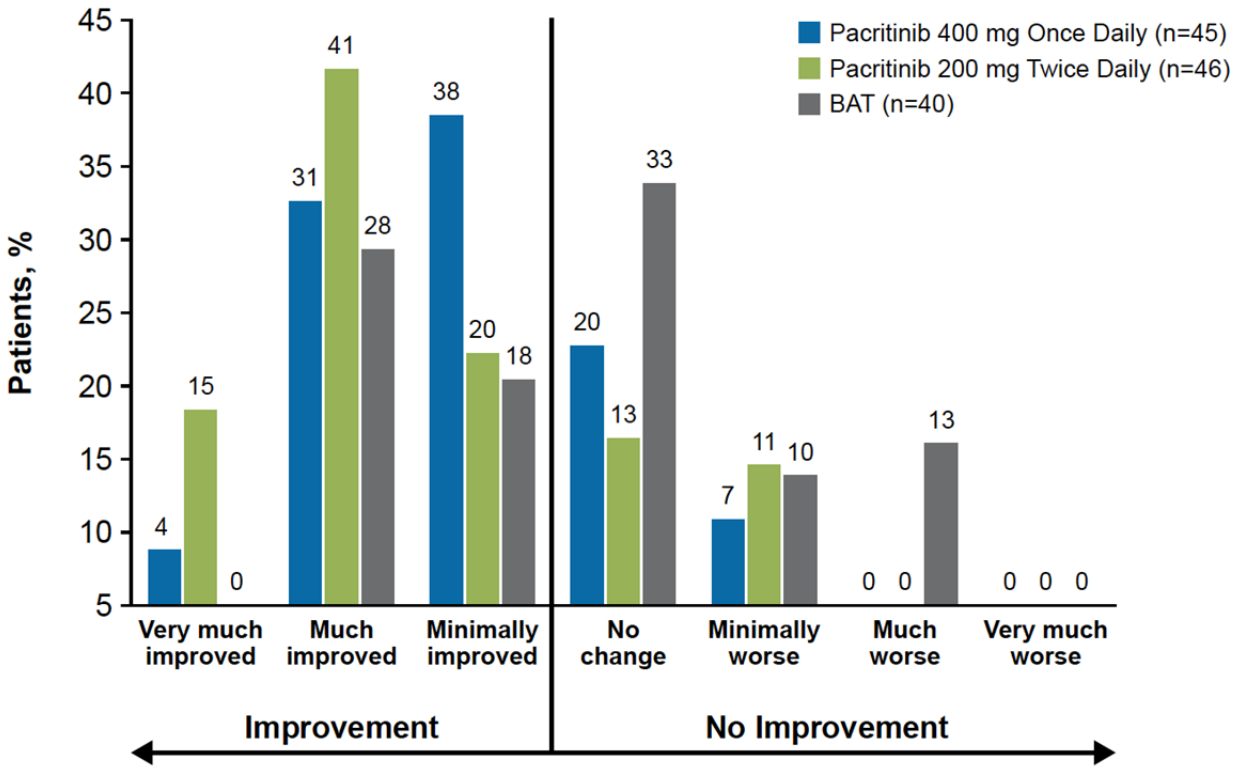


eFigure 1. Proportion of Patients with SVR ≥35% by Subgroup.

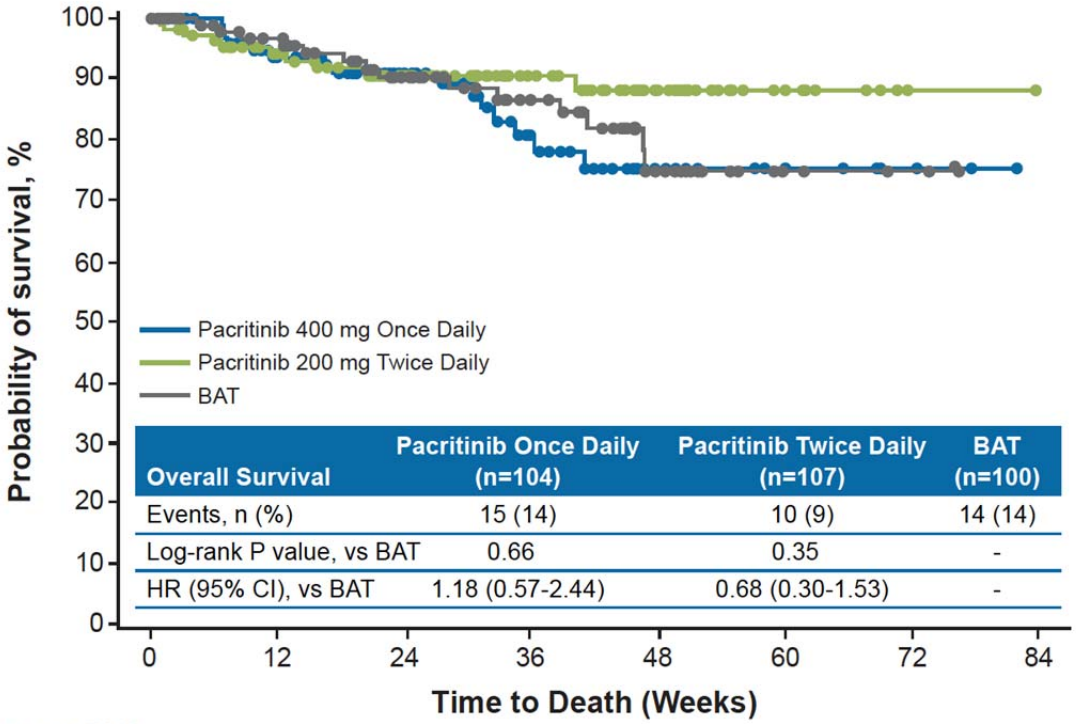
Forest plots of a subgroup analysis of SVR for patients in the pacritinib arms are shown here. Pacritinib patients achieved SVR ≥35% regardless of subgroup, including by sex, age, JAK2^{V617F} mutation status, prior treatment with JAK2 inhibitors, and baseline cytopenias. The overall SVR ≥35% rate with BAT is shown for reference by the vertical line.
Abbreviations: BAT, best available therapy; CI, confidence interval; SVR spleen volume reduction; WBC, white blood cell.



eFigure 2. Changes in Individual Symptom Scores per MPN-SAF TSS 2.0.
Median percent change in individual MPN-SAF TSS 2.0 symptom scores showed greater improvements with pacritinib once daily and twice daily in 7 of 8 (exception of pruritus) and 8 of 8 symptoms, respectively, versus BAT.
Abbreviations: BAT, best available therapy; MPN-SAF TSS 2.0, Myeloproliferative Neoplasm Symptom Assessment Form Total System Score version 2.0.



eFigures 3. Patient Global Impression Assessment (Evaluable Population).
Proportions of patients with much improved or very much improved scores were 36% and 57% with pacritinib once daily and twice daily versus 28% with BAT. Only pacritinib patients achieved very much improved scores, and only BAT patients achieved much worse scores.
Abbrevaition: BAT, best available therapy.

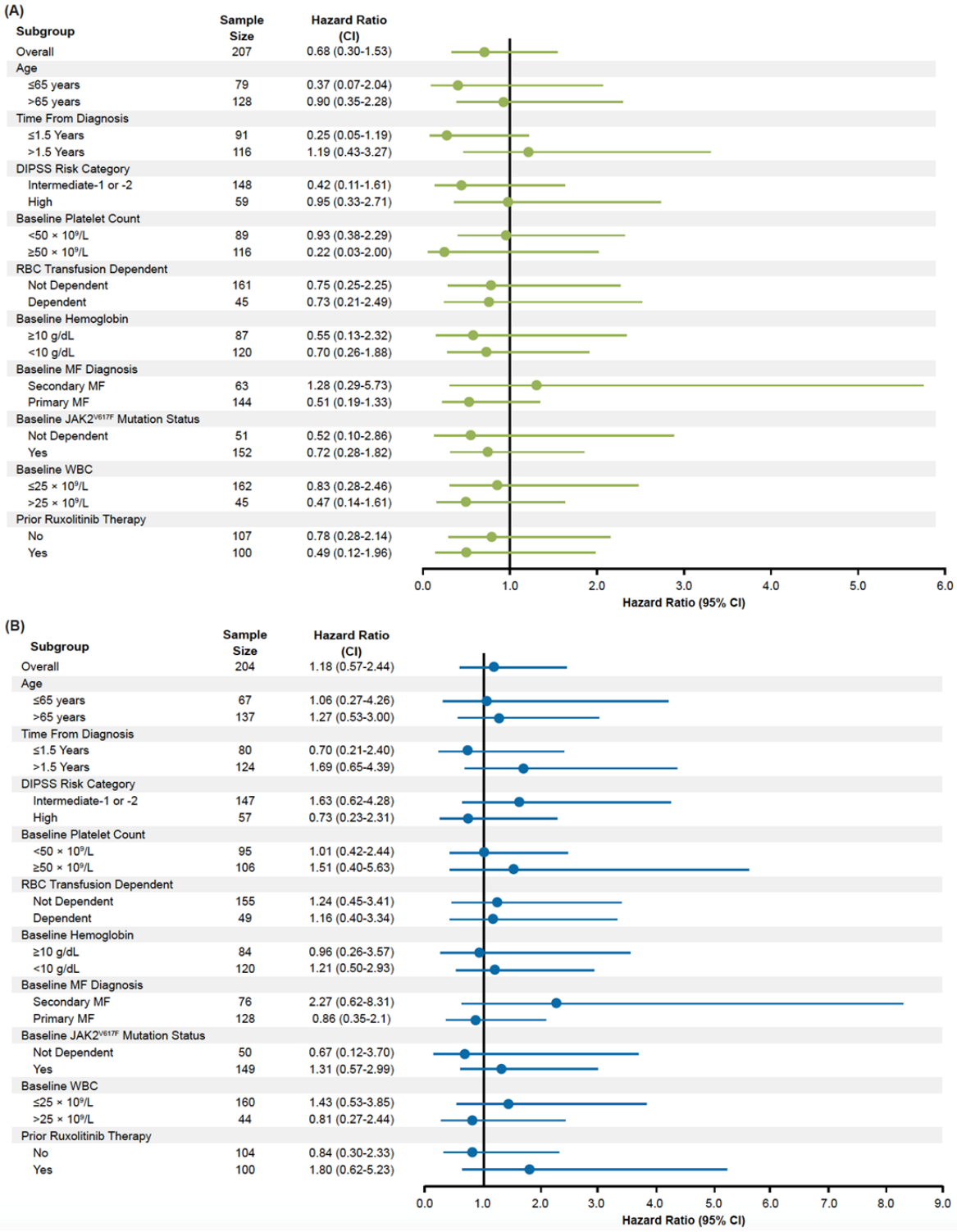


Overall Survival	Pacritinib Once Daily (n=104)	Pacritinib Twice Daily (n=107)	BAT (n=100)
Events, n (%)	15 (14)	10 (9)	14 (14)
Log-rank P value, vs BAT	0.66	0.35	-
HR (95% CI), vs BAT	1.18 (0.57-2.44)	0.68 (0.30-1.53)	-

Patients at Risk, n

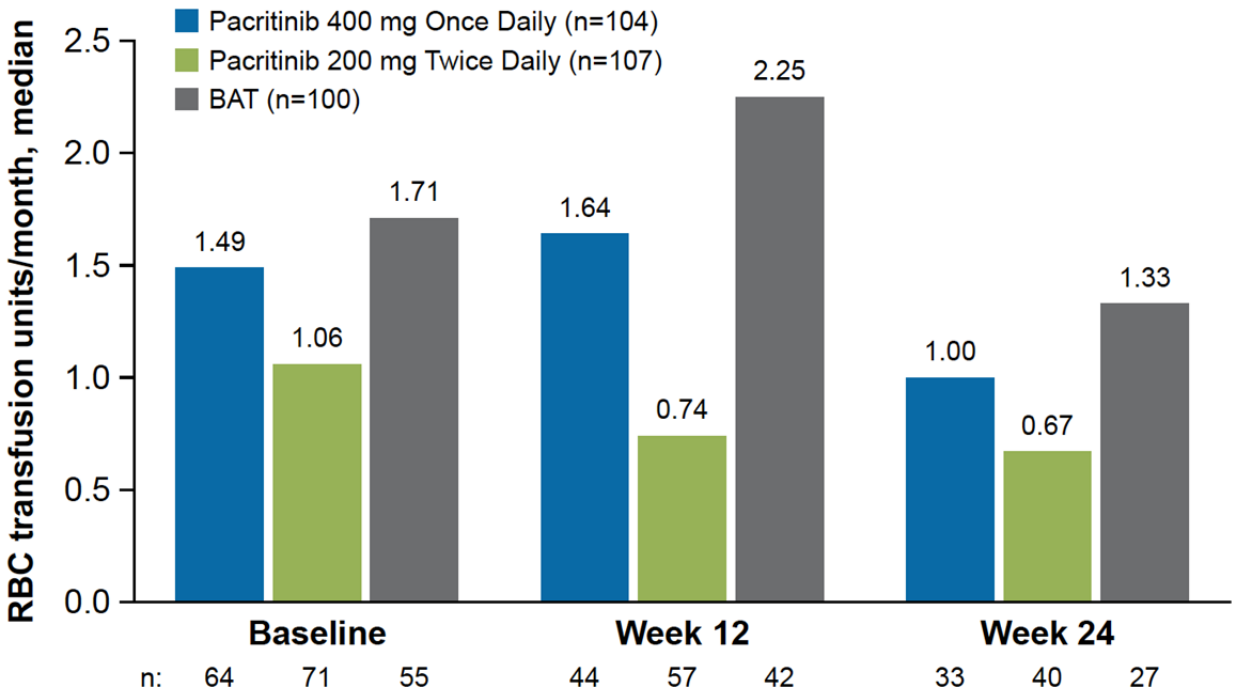
Pacritinib 400 mg Once Daily	104	80	55	31	13	7	3	0
Pacritinib 200 mg Twice Daily	107	85	62	41	22	9	1	0
BAT	100	83	60	41	18	4	2	0

eFigure 4. Overall Survival (Intention-to-Treat, Censored at Date of Clinical Hold).
Kaplan-Meier estimates of overall survival are shown for the 3 treatment arms. Prior to week 24, overall survival was similar across the 3 arms, while after week 24, there was a trend towards improved survival with pacritinib twice daily, though it did not reach statistical significance. Abbrevations: BAT, best available therapy; BID, twice daily; HR, hazard ratio; RBC, red blood cell.



eFigure 5. Subgroup Analysis for Overall Survival (Intention-to-Treat, Censored at Date of Clinical Hold)

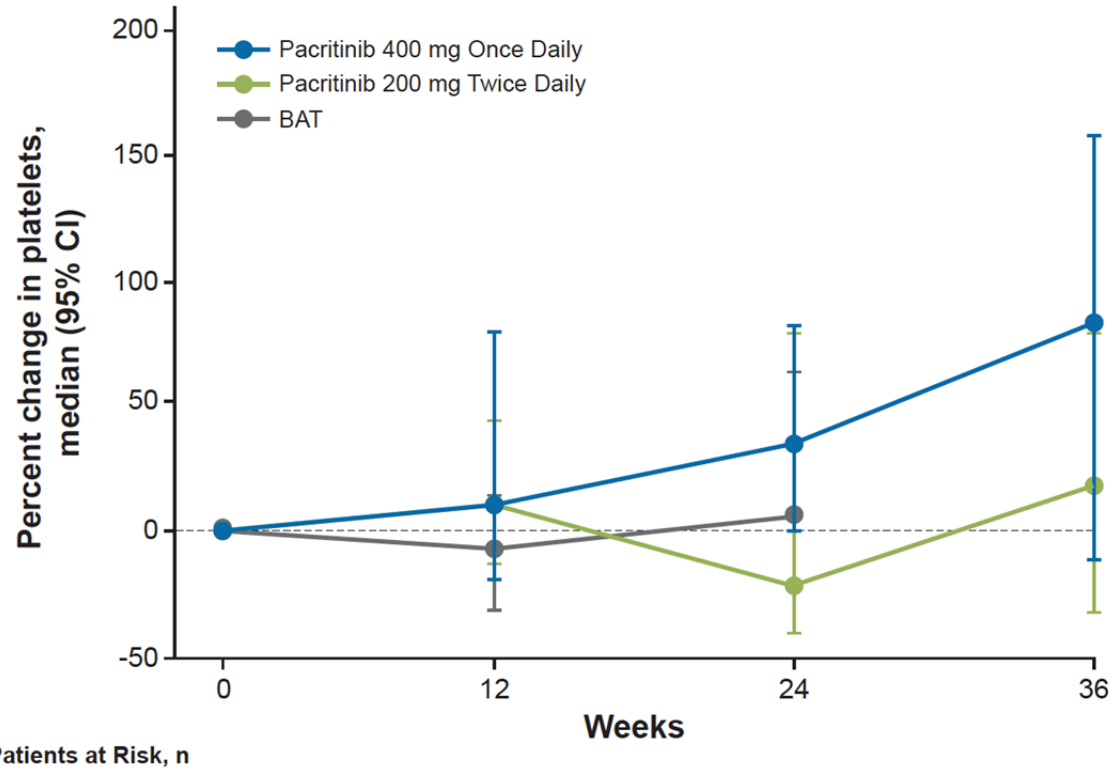
(A) Pacritinib 200 mg Twice Daily or (B) Pacritinib 400 mg Once Daily Versus BAT. Forest plots for various patient subgroups are shown here. Hazard ratios <1 (noted by vertical line) show increased survival with pacritinib versus BAT.
Abbreviations: BAT, best available therapy; CI, confidence interval; DIPSS, Dynamic International Prognostic Scoring System; RBC, red blood cell; MF, myelofibrosis; WBC, white blood cell.



eFigure 6. RBC Transfusions Over Time (Intention-to-Treat).

Median RBC transfusion requirements (units/month) are shown at baseline, week 12, and week 24 for patients on the 3 treatment arms who received ≥1 RBC unit while on study. Patients treated with pacritinib had lower RBC transfusion requirements than those treated with BAT at week 12 and week 24.

Abbreviation: BAT, best available therapy; RBC, red blood cell.



Patients at Risk, n				
Pacritinib 400 mg Once Daily	50	28	19	10
Pacritinib 200 mg Twice Daily	47	32	16	11
BAT	42	23	12	

eFigure 7. Change in Platelets Over Time in Patients With Platelets <50×10^9/L at Baseline.

Median (95% CI) percent change in platelets at weeks 12, 24, and 36 are shown for each of the 3 treatment arms (pacritinib only for week 36). No trends for increasing thrombocytopenia with pacritinib or BAT therapy were noted.

Abbreviations: BAT, best available therapy; CI, confidence interval.