Ruxolitinib *versus* best available therapy in patients with polycythemia vera: 80-week follow-up from the RESPONSE trial

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 doi:10.3324/haematol.2016.143644

Received: February 2, 2016.

Accepted: April 15, 2016.

Pre-published: April 21, 2016.

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Supplemental Material

Ruxolitinib Versus Best Available Therapy in Patients With Polycythemia Vera: 80-Week Follow-Up From the RESPONSE Trial

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Supplemental Methods

Exploratory Analyses

The efficacy of ruxolitinib was evaluated among patients who crossed over to ruxolitinib from the best available therapy arm relative to the arm comprising patients originally randomized to ruxolitinib; these analyses included patients who completed the Week 48 visit or discontinued. The first phlebotomy that occurred in the first 8 weeks was not counted in the calculation of median time to first phlebotomy or exposure-adjusted phlebotomy rate in the ruxolitinib (randomized) arm. In patients receiving ruxolitinib after crossover, the first phlebotomy during the first 8 weeks after crossing over was not counted in calculating median time to first phlebotomy. Changes in spleen volume and blood counts after 32 weeks of study treatment were based on the original Baseline value for the randomized treatment arms and in the crossover treatment group.

*JAK2*V617F allele burden was assessed at Baseline and Weeks 32 and 80 in the ruxolitinib and best available therapy arms, as well as at the date of crossover to ruxolitinib and 48 weeks after crossover to ruxolitinib.

Statistical Analyses

The statistical methods used for primary study analyses were described previously.[1] Analysis of change from Baseline in CHR at Week 32 was performed using the Cochran-Mantel-Haenszel test (1) adjusted for abnormal versus normal white blood cell and/or platelet status at Baseline (abnormal status defined as white blood cell count $>15\times10^9$/L and/or platelet count $>600\times10^9$/L) and (2) unadjusted. All new data in the current analysis were summarized using descriptive statistics.

Supplementary Table

Supplementary Table 1. Spleen volume change from original Baseline by percent reduction after 32 weeks on treatment

Proportion of Patients, n (%)	Ruxolitinib (n=110)	Ruxolitinib Crossover (n=96)	Best Available Therapy (n=112)
Increase in spleen volume	17 (15.5)	11 (11.5)	50 (44.6)
0 to <10% reduction	4 (3.6)	9 (9.4)	16 (14.3)
10% to <35% reduction	33 (30.0)	21 (21.9)	20 (17.9)
≥35% reduction	44 (40.0)	18 (18.8)	1 (0.9)

Supplementary Figures

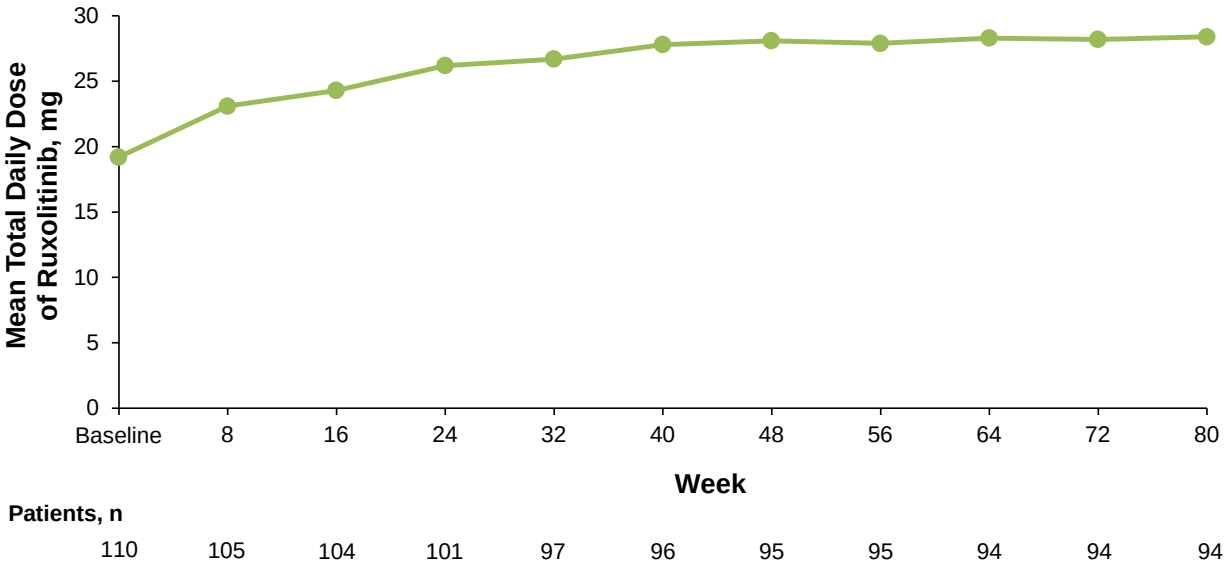
Supplementary Figure 1. Mean daily dose of ruxolitinib over time.

Supplementary Figure 2. Distribution of ruxolitinib dosing at Week 32 and Week 80.

Supplementary Figure 3. Change in spleen volume from Baseline over time. *Data after crossover to ruxolitinib are excluded; only visits with data from >5 patients are included.

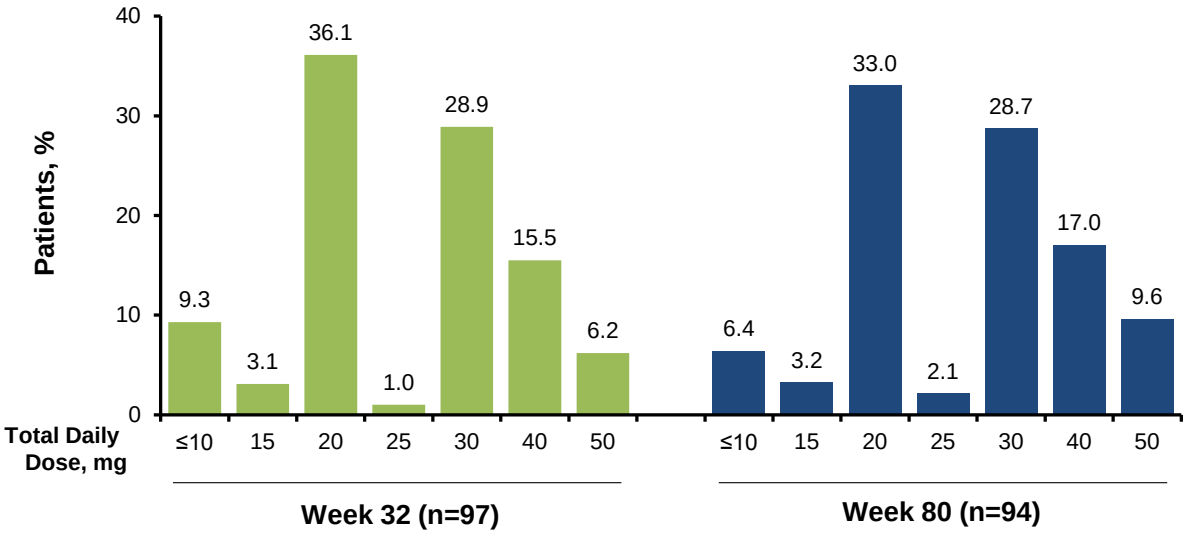
Supplementary Figure 4. Individual changes in spleen volume from original Baseline after 16 weeks on treatment.

Supplementary Figure 1. Mean daily dose of ruxolitinib over time

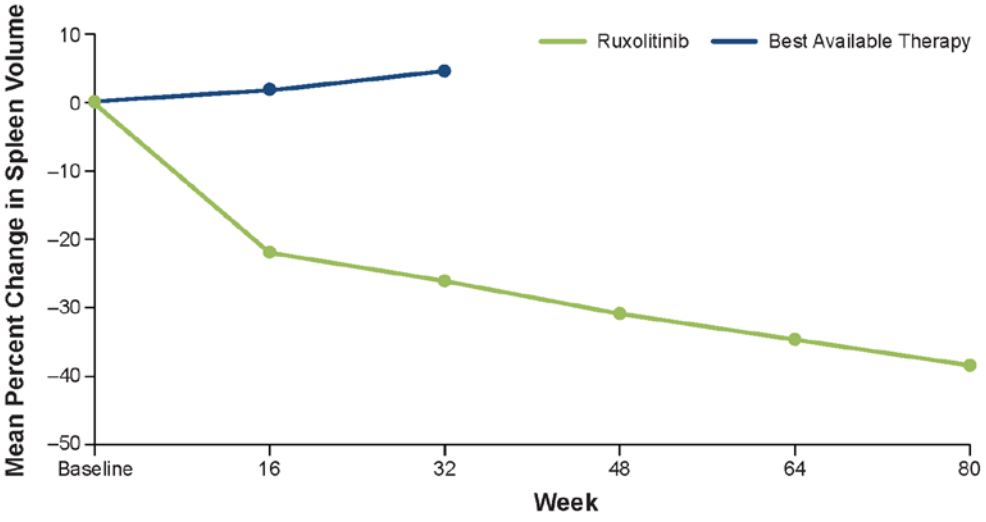


Patients, n										
110	105	104	101	97	96	95	95	94	94	94

Supplementary Figure 2. Distribution of ruxolitinib dosing at Week 32 and Week 80

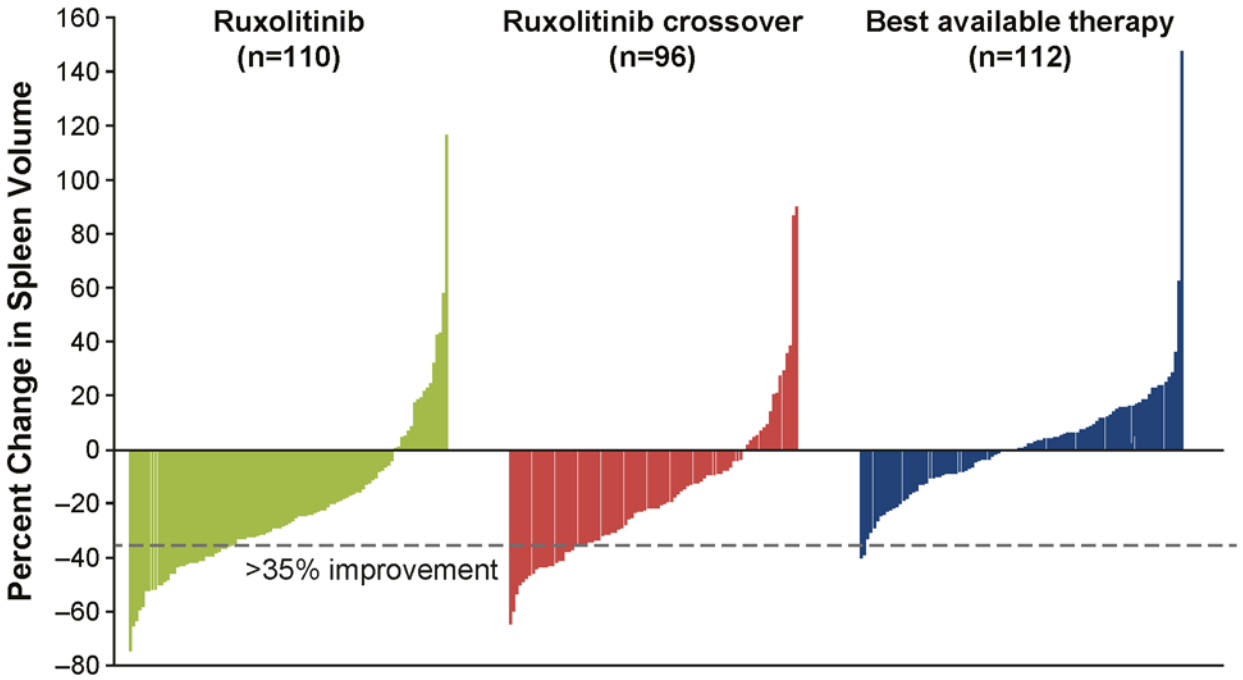


Supplementary Figure 3. Change in spleen volume from Baseline over time



Ruxolitinib, n	110	100	96	89	91	91
Best Available Therapy, n	109	103	87			

Supplementary Figure 4. Individual changes in spleen volume from original Baseline after 16 weeks on treatment



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