Retrospective Analysis of the Relationship between Transfusion Independence and Bone Marrow Fibrosis Reduction in Patients with Myelofibrosis Treated with Pacritinib versus Ruxolitinib

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CONCLUSIONS

• In cytopenic myelofibrosis (MF) patients from PERSIST-2, transfusion independence (TI) response was seen in 28% of those treated with PAC compared to 11% of those treated with RUX (P = 0.043).

• Patients treated with PAC had a significantly greater proportion of patients achieving≥50% transfusion reduction than those on RUX (30% vs. 19%; P = 0.023). This provides additional evidence for the correlation between fibrosis reduction and TI in MF, and reaffirms the importance of antifibrotic therapy in improving outcomes for these patients.

• This analysis also highlighted that children with baseline platelets ≥50 x 10^9/L had a significantly greater proportion of patients achieving TI compared to those with lower platelet counts (P = 0.001).

METHODS

• This study included patients who received PAC 200 mg twice daily (BID) vs RUX as best available therapy (BAT) on the phase 3 PERSIST-2 study.

• TI was assessed using miniature criteria.

• To be considered TI, patients did not receive any red blood cell transfusions for ≥12 weeks.

• Treatment comparisons (PAC vs. RUX) for efficacy outcomes, TI and ≥50% transfusion reduction in transfusion burden were performed using the Fisher’s exact test.

AIM

• To retrospectively analyze the relationship between achieving transfusion independence (TI) and reduction in bone marrow fibrosis in MF patients treated with PAC 200 mg twice daily (BID) vs RUX.

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RESULTS

• Baseline characteristics were similar between the PAC and RUX groups, including median platelet count (41 vs 38 x 10^9/L) and median hemoglobin (8.7 vs 8.6 g/dL, Table 1).

• All patients required RBC transfusion at baseline.

• This analysis focused on patients who received PAC 200 mg BID and RUX, using the Fisher’s exact test.

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• The proportion of patients on PAC who experienced bone marrow fibrosis reduction ≥1 grade decrease in reticulin fibrosis from baseline at week 24 was reported among patients on PAC achieving TI vs. non-response (NR).

• Greater proportion of patients on pacritinib achieve TI on RUX as best available therapy (BAT) on the phase 3 PERSIST-2 study. This analysis focused on patients who received PAC 200 mg BID and RUX, using the Fisher’s exact test.

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• The proportion of patients on PAC who experienced bone marrow fibrosis reduction ≥1 grade decrease in reticulin fibrosis from baseline at week 24 was reported among patients on PAC achieving TI vs. non-response (NR).

• A significantly greater proportion of patients treated with PAC vs RUX achieved TI through week 24: 37% (n=15/41) vs 6% (n=1/18), P=0.023.

• Nominally, this trend held for those with baseline platelets ≤50 x 10^9/L: 28% vs 8%, P=0.222 (Figure 3).

• A significantly greater proportion of patients treated with PAC vs RUX achieved ≥50% transfusion reduction in RBC transfusions over any 12 weeks for all patients (49% vs 6%, P=0.001) and those with baseline platelets <50 x 10^9/L (40% vs 8%, P=0.06) (Figure 4).

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• The proportion of patients on PAC who experienced bone marrow fibrosis reduction ≥1 grade was significantly greater among TI-responders (62.5% vs. 56%, P=0.043) compared to non-TI responders (20%, n=1/5) (Figure 5).

• Of the 5 patients who achieved TI response and bone marrow fibrosis reduction, all had grade 2–3 fibrosis at baseline and 2 experienced a reduction from grade 3 to grade 1.

• By contrast, paired bone marrow biopsies were available for 5 patients on RUX (2 patients had baseline bone marrow fibrosis stage 3, 2 patients had stage 2, and 1 patient had stage 1), and there was no association between fibrosis reduction in TI responders (0%, n=0/5) and non-responders (25%, n=1/4).

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