OUTCOMES IN PATIENTS WITH MYELOFIBROSIS AND RBC-TRANSFUSION DEPENDENCE IN THE PHASE III PERSIST-1 STUDY VS. BEST AVAILABLE THERAPY

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Abstract 7066

INTRODUCTION

- A phase 3 trial evaluated pacritinib, a multi-targeted receptor tyrosine kinase inhibitor, in patients with myelofibrosis (MF) who had received prior treatment with JAK2 inhibitors, with the primary endpoints of spleen reduction and Hb increase

METHODS

- Patients in the PERSIST-1 trial were randomized to treatment with pacritinib or placebo
- Key Eligibility Criteria:
  - Median age ≥18 years
  - A diagnosis of primary MF, post-polycythemia vera MF, or post-essential thrombocythemia MF
  - A baseline spleen length ≥5 cm
  - A hemoglobin level of 9.0-11.5 g/dL
  - A platelet count of ≥50,000/μL and <100,000/μL

RESULTS

- Characteristics of Patients With and Without RBC-TD at Baseline:
  - Among pacritinib-treated patients, 36 (20%) were RBC-TD and 19 (10.5%) were not at baseline
  - Median spleen length by physical exam was 12 cm (range 5-40 cm)

RESULTS (Table 1 shows baseline characteristics of patients receiving pacritinib or placebo) (as of Week 24)

- Table 1. Baseline Characteristics

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>PAC (n=46)</th>
<th>PL (n=25)</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Male, n (%)</td>
<td>31 (67%)</td>
<td>13 (52%)</td>
<td>.06</td>
</tr>
<tr>
<td>Median age, years (range)</td>
<td>66 (53-84)</td>
<td>61 (46-82)</td>
<td>.68</td>
</tr>
<tr>
<td>Median Platelet Count, 10^9/µL</td>
<td>116 (50-200)</td>
<td>131 (50-200)</td>
<td>.84</td>
</tr>
<tr>
<td>Median Hemoglobin, g/dL</td>
<td>10.8 (9.0-11.5)</td>
<td>10.8 (9.0-11.5)</td>
<td>1.00</td>
</tr>
<tr>
<td>Median platelet count at Week 24, 10^9/µL</td>
<td>124 (40-220)</td>
<td>130 (50-220)</td>
<td>.36</td>
</tr>
<tr>
<td>Median spleen length cm (range)</td>
<td>12 (5-40)</td>
<td>12 (5-40)</td>
<td>1.00</td>
</tr>
<tr>
<td>Median percent reduction in spleen volume from baseline to Week 24 (%)</td>
<td>30 (10-60)</td>
<td>15 (0-40)</td>
<td>.006</td>
</tr>
<tr>
<td>Median percent reduction in Hb from baseline to Week 24 (%)</td>
<td>2.5 (0-7)</td>
<td>1.0 (0-5)</td>
<td>.007</td>
</tr>
</tbody>
</table>

- Patient Disposition (Patients With Baseline RBC-TD):
  - 12 of 54 (22%) RBC-TD patients treated with pacritinib either from study start or crossover period

- Changes in Hemoglobin
  - Median Hb increase from baseline to Week 72 was 3.8 g/dL with pacritinib

- Impact of Reduced Transfusion Burden on Patient Outcomes:
  - 23 of 36 (64%) patients were RBC-TD at baseline, 81% and 62% achieved RBC-TD, respectively, at baseline and Week 24

CONCLUSIONS

- Pacritinib treatment resulted in achievement of RBC-TD in 22% of patients
- Pacritinib-treated patients demonstrated clinically meaningful reductions in spleen volume and hemoglobin levels compared to placebo

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References


No competing interests declared.