Clinical benefit of luspatercept in patients with lower-risk myelodysplastic syndromes and high transfusion burden in the phase 3 MEDALIST study

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Study endpoints

• Achievement of HI-E per IWG 2006 criteria
• Average RBC transfusion burden from baseline over at least 24 weeks

Patients receiving transfusion of RBC units within 6 weeks prior to study entry. HTB, high transfusion burden; LTB, low transfusion burden; RBC-TI, red blood cell transfusion independence.

Table 1. Baseline characteristics

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Luspatercept (n = 15)</th>
<th>Placebo (n = 76)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, mean (SD), y</td>
<td>60 (17)</td>
<td>60 (17)</td>
</tr>
<tr>
<td>Sex, n (%)</td>
<td>10 (67)</td>
<td>61 (80)</td>
</tr>
<tr>
<td>Baseline transfusion burden (median), RBC units</td>
<td>18 (15–48)</td>
<td>18 (15–48)</td>
</tr>
<tr>
<td>Baseline transfusion burden (mean), RBC units</td>
<td>21.6 (15.2)</td>
<td>21.6 (15.2)</td>
</tr>
<tr>
<td>Baseline EPO level, mean, IU/L</td>
<td>48 (40)</td>
<td>57 (50)</td>
</tr>
<tr>
<td>Baseline Hb level, mean, g/dL</td>
<td>9.9 (1.2)</td>
<td>10.0 (1.2)</td>
</tr>
<tr>
<td>WHO stage, n (%)</td>
<td>11 (74)</td>
<td>71 (93)</td>
</tr>
<tr>
<td>WHO performance status, mean</td>
<td>1.3 (0.6)</td>
<td>1.3 (0.6)</td>
</tr>
<tr>
<td>Number of prior RBC units transfused, median</td>
<td>11 (4–75)</td>
<td>11 (4–75)</td>
</tr>
</tbody>
</table>

Average RBC transfusion burden

- ESA-naive: EPO > 200 U/L or ≥ 5% with erythropoiesis-stimulating agents (ESAs)
- ESA-experienced: EPO > 200 U/L or ≥ 5% with erythropoiesis-stimulating agents (ESAs)

Dose titrated up to a maximum of 1.75 mg/kg

Safety

- Dose titrated up to a maximum of 1.75 mg/kg
- Randomized 2:1
- Patients followed ≥ 3 years post final dose
- Crossover between groups was not allowed
- In the placebo arm, as of the data cutoff, all patients had discontinued treatment

In the placebo arm, as of the data cutoff, all patients had discontinued treatment

1 of 1 patients who achieved RBC-TI with placebo

In Weeks 1–48, 37 of 66 (56.1%) HTB patients in the luspatercept arm and 9 of 33 (27.3%) HTB patients in the placebo arm achieved the primary endpoint of RBC-TI

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Reducition in transfusion burden

- 50% reduction in RBC transfusion burden from baseline over at least 24 weeks

Figure 4. Patients achieving a 50% (A) and a 75% (B) reduction in RBC transfusion burden from baseline over at least 24 weeks

Conclusions

• In this study of patients with LR MDS with RS, luspatercept treatment resulted in clinically significant reductions in RBC transfusion burden and achievement of HTB, including in HTB patients
• Although rates of RBC-TI in Weeks 1–24 were low in HTB patients receiving luspatercept, there were reductions in RBC transfusion events, and HTB patients had longer periods of transfusion independence

References


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Disclosures

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