**INTRODUCTION**

MDS is a clonal hematopoietic stem cell disorder characterized by ineffective erythropoiesis, which leads to anemia and red blood cell (RBC) transfusion dependence.

Additional cytopenias in patients with lower RBC MDS can impair quality of life and increase the risk of adverse outcomes.

Effectiveness treatments for anemia in transfusion-dependent patients with lower RBC MDS are lacking due to an unmet clinical need.

**OBJECTIVE**

To report hematologic improvement (HI) outcomes for patients in the MEDALIST trial.

**METHODS**

**TREATMENT**

MEDALIST is a phase 3, randomized, double-blind, placebo-controlled study of luspatercept to treat anemia in patients with RBC transfusion burden of <4 units/8 weeks.

**PATIENTS**

Patients were randomized between March 2016 and June 2017 at 155 centers in 19 countries.

**RESULTS**

**HI Endpoints**

- **HI-E** was achieved in 30 of 153 (20.0%) patients in the luspatercept arm and 1 of 76 (1.3%) patients in the placebo arm during Weeks 1–48 (Table 2).

- Patients were evaluable for HI-E during Weeks 1–24 (121/153 [79.3%] in the luspatercept arm, 66/76 [86.8%] in the placebo arm).

- Of patients evaluable for HI-N (n = 25), 20.0% achieved HI-N in the luspatercept arm (Table 3).

**CHANGES IN PLATELETS**

- By Cycle 5, Day 6, mean change from baseline in platelets was 108 × 10^9/L in the luspatercept arm and 85 × 10^9/L in the placebo arm (Figure 1).

- Early increases were observed in the luspatercept arm by Cycle 1, Day 8 (0.88 × 10^9/L vs. 0.08 × 10^9/L for placebo).

- The mean increase in platelets during Weeks 1–48 was achieved by 37.9% of patients in the luspatercept arm (n = 148) compared with 11.1% in the placebo arm (Figure 1).

- Of patients evaluable for HI-N (baseline neutrophil count < 1 × 10^9/L), 10.0% achieved HI-N in the luspatercept arm (Table 3).

**CONCLUSIONS**

A greater proportion of patients in the luspatercept arm achieved HI-E during Weeks 1–48 than in the placebo arm (88.6% vs. 17.1%).

A greater proportion of patients in the luspatercept arm achieved HI-N during Weeks 1–48 versus 10.0% in the placebo arm.

HI-N endpoints were achieved in 57% of patients in the luspatercept arm and 16% in the placebo arm.

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**REFERENCES**


**DISCLOSURES**

All authors have reported no potential conflicts of interest.

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**Figure 1**

Mean change from baseline in neutrophil count (n = 153).

**Table 1**

Patients achieving HI-E, HI-N, and HI-P (n = 153).

**Table 2**

Patients achieving HI-E, HI-N, and HI-P (n = 153).

**Table 3**

Patients achieving HI-N (n = 25).

**Figure 2**

Hematologic Toxicities

- **Treatment-emergent grade 4 or 5 neutropenia was reported in 15 patients (7.6%) patients receiving luspatercept and 6 (7.9%) receiving placebo**

- **No grade 3 or 4 treatment-emergent thrombocytopenia was reported in the luspatercept arm during Weeks 1–48**

- **1 patient in the luspatercept arm who achieved HI-N or HI-P progressed to acute myeloid leukemia**

- **None of the patients who achieved HI-N or HI-P progressed to acute myeloid leukemia**

- **Thrombocytopenia**

- **Neutropenia**

- **Anemia**

**Table 4**

Baseline and HI-E, HI-N, HI-P, and baseline parameters (n = 153).

**Table 5**

Baseline and HI-E, HI-N, HI-P, and baseline parameters (n = 153).

**Table 6**

Baseline and HI-E, HI-N, HI-P, and baseline parameters (n = 153).