

Eltrombopag Added to Immunosuppression in Severe Aplastic Anemia

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CLINICAL PROBLEM

Standard therapy for severe aplastic anemia that entails immunosuppression with horse antithymocyte globulin (ATG) plus cyclosporine elicits a response in over two thirds of patients. A single-group, phase 1–2 study suggested that adding eltrombopag, an oral thrombopoietin-receptor agonist, to this regimen could improve outcomes, but additional research was needed.

CLINICAL TRIAL

Design: A phase 3, prospective, open-label, multi-center, randomized trial examined the efficacy and safety of standard immunosuppressive therapy, either alone or with eltrombopag, as first-line treatment in patients with severe aplastic anemia.

Intervention: 197 untreated patients (≥15 years of age) with newly diagnosed severe aplastic anemia were assigned to receive intravenous horse ATG plus oral cyclosporine, with or without oral eltrombopag. The primary end point was hematologic complete response at 3 months, defined as a hemoglobin level >10 g per deciliter, an absolute neutrophil count >1000 per cubic millimeter, and a platelet count >100,000 per cubic millimeter in patients who had not received transfusions.

RESULTS

Efficacy: The percentage of patients with a hematologic complete response at 3 months was significantly higher with eltrombopag. Overall response (complete or partial response) at 6 months also favored eltrombopag.

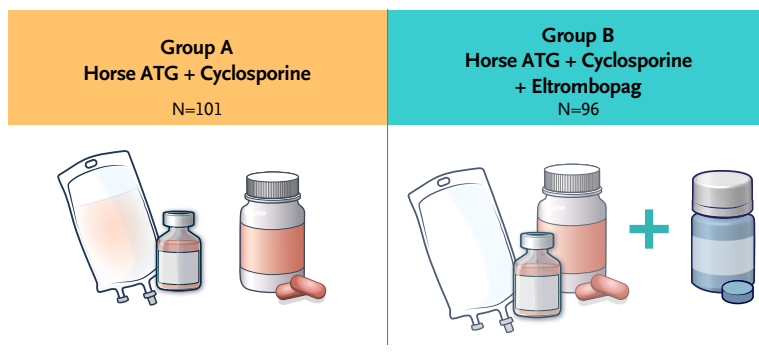
Safety: The incidence of all adverse events, including infectious and hepatic complications, was similar in the two treatment groups.

LIMITATIONS AND REMAINING QUESTIONS

Further study is required to understand the following:

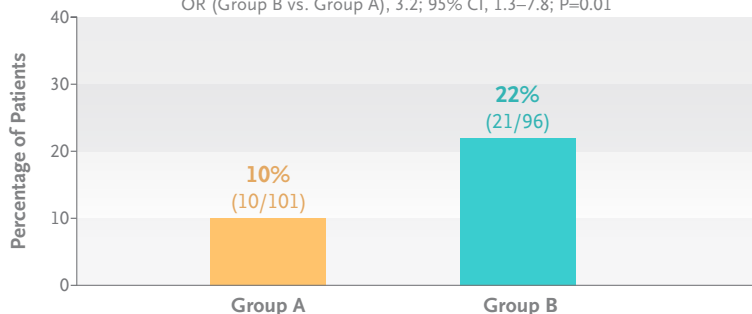
- The mechanisms of action of eltrombopag in patients with aplastic anemia.
- Long-term treatment in patients with aplastic anemia.

Links: [Full Article](#) | [NEJM Quick Take](#)

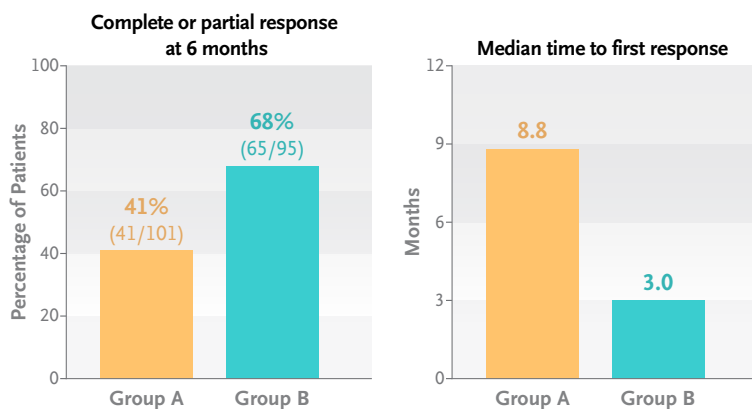


Hematologic Complete Response at 3 Months

OR (Group B vs. Group A), 3.2; 95% CI, 1.3–7.8; P=0.01



Secondary End Points



CONCLUSIONS

Among patients with previously untreated severe aplastic anemia, the percentage of patients with a hematologic complete response at 3 months was higher among those who received eltrombopag plus standard immunosuppressive therapy than among those who received standard therapy alone, without additional toxic effects.