Luspatercept mitigates anemia in lower-risk myelodysplastic syndromes
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For patients with lower-risk myelodysplastic syndromes with ring sideroblasts who have been receiving regular red-cell transfusions, luspatercept reduces the severity of anemia compared with placebo, according to a study published in the Jan. 9 issue of the *New England Journal of Medicine*.

Pierre Fenaux, M.D., Ph.D., from the Hôpital Saint-Louis in Paris, and colleagues enrolled 229 patients with very low-risk, low-risk, or intermediate-risk myelodysplastic syndromes with ring sideroblasts who had been receiving regular red-cell transfusions. Patients were randomly assigned to receive either luspatercept (at a dose of 1.0 to 1.75 mg/kg body weight; 153 patients) or placebo (76 patients) every three weeks.

The researchers found that transfusion independence for eight weeks or longer was observed in 38 and 13 percent of patients in the luspatercept and placebo groups, respectively. The key secondary end point (transfusion independence for 12 weeks or longer) was met by a higher percentage of patients in the luspatercept group versus the placebo group (28 versus 8 percent for weeks 1 through 24; 33 versus 12 percent for weeks 1 through 48). Fatigue, diarrhea, asthenia, nausea, and dizziness were the most common luspatercept-associated adverse events; over time, there was a decrease in the incidence of adverse events.

"Luspatercept significantly reduced the transfusion burden in a substantial proportion of these patients and was associated with mainly low-grade toxic effects," the authors write.

Several authors disclosed ties to the pharmaceutical industry, including Celgene and Acceleron Pharma, which manufacture luspatercept and partially funded the study.

More information: Abstract/Full Text (subscription or payment may be required)