Imetelstat leads to durable red blood cell transfusion independence

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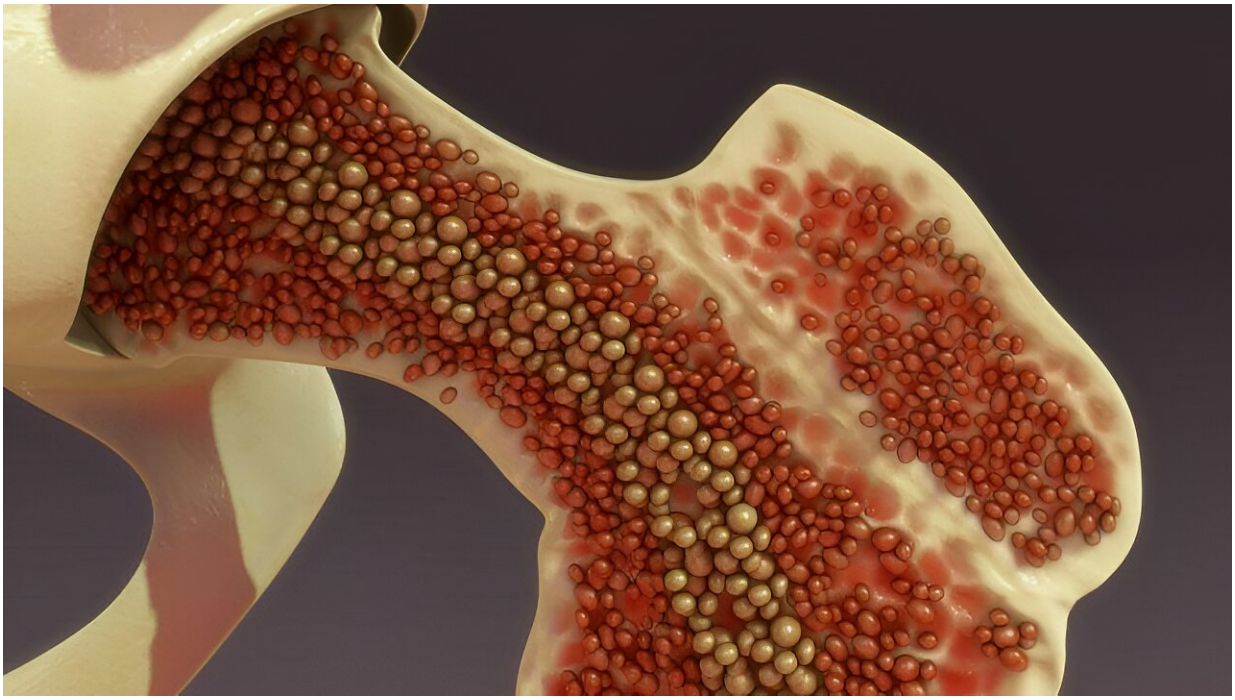

For heavily transfused patients with lower-risk myelodysplastic syndromes (LR-MDS) who are not responding to or are ineligible for erythropoiesis-stimulating agents (ESAs), the competitive telomerase inhibitor imetelstat leads to durable red blood cell (RBC) transfusion independence, according to a study published online Dec. 1 in *The Lancet*.

Uwe Platzbecker, M.D., from University Hospital Leipzig in Germany, and colleagues compared the RBC transfusion independence rate with imetelstat versus placebo in patients with RBC transfusion-dependent LR-MDS in a double-blind trial conducted at 118 sites. Overall, 178 patients (aged 18 years and older) with ESA-relapsed, ESA-refractory, or ESA-ineligible LR-MDS were enrolled and randomly assigned to imetelstat or placebo (118 and 60, respectively), administered every four weeks as a two-hour intravenous infusion.

Patients were followed for a median of 19.5 and 17.5 months in the imetelstat and placebo groups, respectively. The researchers found that 40 and 15 percent of patients had an RBC transfusion independence of at least eight weeks in the imetelstat and placebo groups, respectively. Of the patients receiving imetelstat and placebo, 91 and 47 percent, respectively, had grade 3 to 4 treatment-emergent adverse events, with the most common events being neutropenia and thrombocytopenia for those taking imetelstat. There were no reports of treatment-related deaths.

"Taken together, IMerge phase 3 results validate the observations from the phase 2 part and show that imetelstat provides clinically significant benefit to a heavily transfusion-dependent LR-MDS patient population," the authors write.

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More information: Uwe Platzbecker et al, Imetelstat in patients with lower-risk myelodysplastic syndromes who have relapsed or are refractory to erythropoiesis-stimulating agents (IMerge): a multinational, randomised, double-blind, placebo-controlled, phase 3 trial, *The Lancet* (2023). DOI: 10.1016/S0140-6736(23)01724-5

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