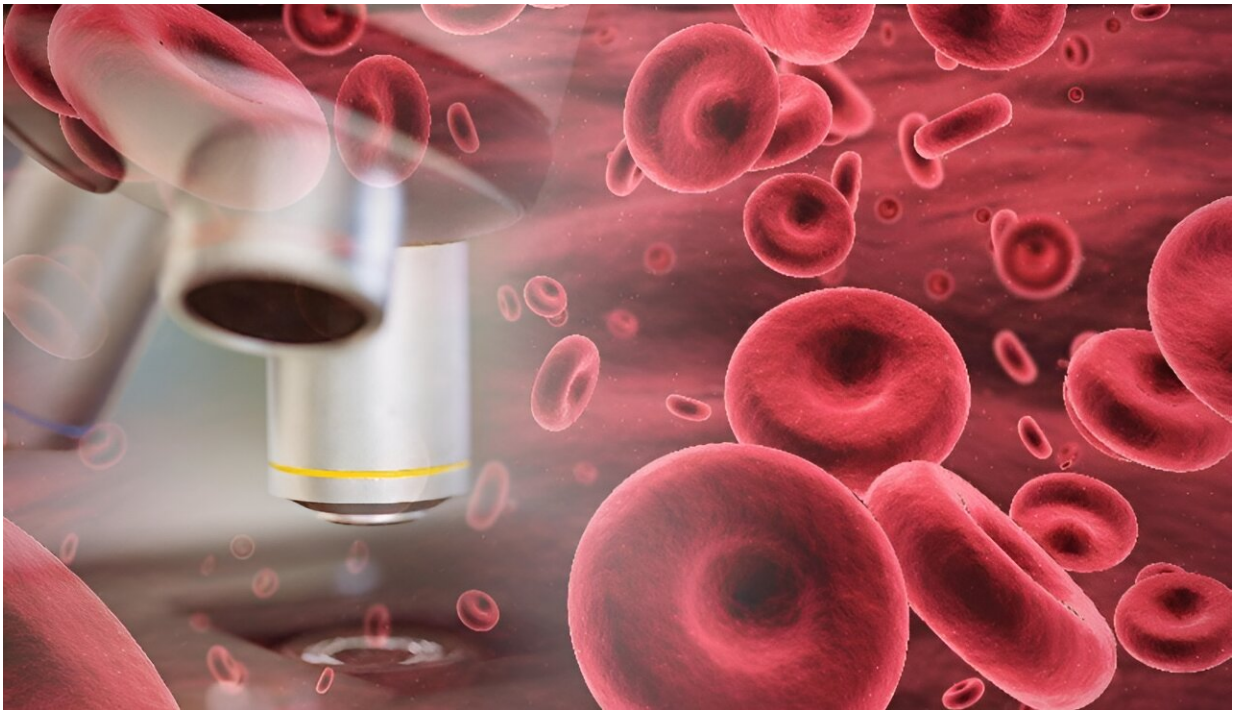


Iptacopan improves hematologic, clinical outcomes in persistent anemia

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The first-in-class oral factor B inhibitor iptacopan improves hematologic and clinical outcomes in anti-C5-treated patients with persistent anemia and in those who have not received complement inhibitors, according to a study published in the March 14 issue of the *New England Journal of Medicine*.

Régis Peffault de Latour, M.D., from Saint-Louis Hospital in Paris, and colleagues conducted two phase 3 [trials](#) to assess iptacopan monotherapy during a 24-week period in [patients](#) with [hemoglobin levels](#) of less than 10 g/dL. In the first study, anti-C5-treated patients were randomly assigned to continue anti-C5 therapy or switch to iptacopan, and in the second single-group trial, patients who had not received complement inhibitors and with lactate dehydrogenase levels >1.5 times the upper limit of the normal range received iptacopan.

The researchers found that in the first trial, 51 of 60 patients receiving iptacopan had an increase of ≥ 2 g/dL from baseline in the hemoglobin levels and 42 had a hemoglobin level of at least 12 g/dL, both without red-cell transfusions; none of the anti-C5-treated patients attained the end-point levels. In the second trial, 31 of 33 patients had an increase ≥ 2 g/dL from baseline in the hemoglobin levels without red-cell transfusion.

No transfusion was received by 59 of 62 patients receiving iptacopan or 14 of 35 patients receiving anti-C5 treatment in the first trial, nor by any patients in the second trial. Hemoglobin levels were increased, fatigue was reduced, and reticulocyte and bilirubin levels were reduced with iptacopan [treatment](#).

"Blocking the complement system proximally at the alternative pathway with monotherapy with an oral factor B inhibitor was effective and safe and did not lead to the use of additional combined terminal blockade," the authors write.

More information: Régis Peffault de Latour et al, Oral Iptacopan Monotherapy in Paroxysmal Nocturnal Hemoglobinuria, *New England Journal of Medicine* (2024). [DOI: 10.1056/NEJMoa2308695](https://doi.org/10.1056/NEJMoa2308695)

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