Medical Xpress

Efgartigimod efficacious, safe for primary immune thrombocytopenia

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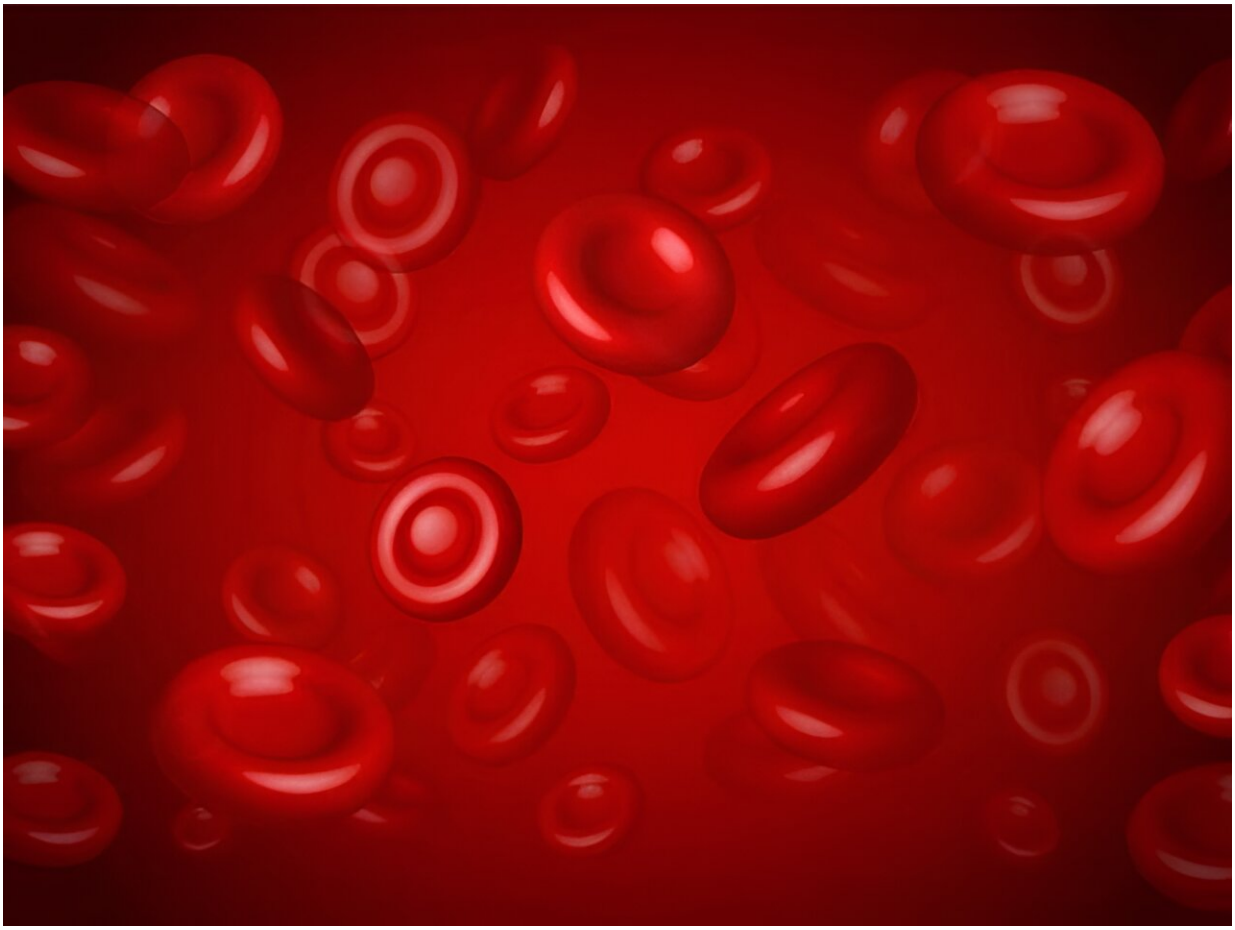

The first-in-class novel human immunoglobulin G1 Fc fragment,

efgartigimod, is efficacious and safe for patients with chronic or persistent primary immune thrombocytopenia, according to a study published online Sept. 28 in *The Lancet*.

Catherine M. Broome, M.D., from Georgetown University in Washington, D.C., and colleagues conducted a phase 3 24-week study to assess the efficacy and safety of intravenous efgartigimod in adults aged 18 years or older with chronic or persistent primary immune thrombocytopenia who had an average platelet count of less than 30,000, had responded to at least one previous immune thrombocytopenia therapy, and were on or had received at least a second previous immune thrombocytopenia therapy. A total of 131 patients were randomly assigned to efgartigimod or placebo (86 and 45, respectively).

The researchers found that 22 and 5 percent of those receiving efgartigimod and placebo, respectively, reached the primary end point of sustained platelet count response. The median number of weeks of disease control was 2.0 and 0.0 for patients with chronic immune thrombocytopenia receiving efgartigimod and placebo, respectively. Efgartigimod was well tolerated, with adverse events mostly of mild-to-moderate severity. In both groups, the most common adverse events of interest were headache, hematuria, and petechiae.

"The higher proportion of efgartigimod-treated patients with sustained platelet count responses and improved disease control compared with placebo in this population with chronic and persistent disease corroborates and extends the evidence of this novel mechanism for targeting the autoantibody-based pathophysiology of immune thrombocytopenia," the authors write.

Several authors disclosed ties to biopharmaceutical companies, including argenx, which manufactures efgartigimod and funded the study.

More information: Catherine M Broome et al, Efficacy and safety of the neonatal Fc receptor inhibitor efgartigimod in adults with primary immune thrombocytopenia (ADVANCE IV): a multicentre, randomised, placebo-controlled, phase 3 trial, *The Lancet* (2023). DOI: 10.1016/S0140-6736(23)01460-5

Syed Mahamad et al, Inhibition of neonatal Fc receptor as a treatment for immune thrombocytopenia, *The Lancet* (2023). DOI: 10.1016/S0140-6736(23)01836-6