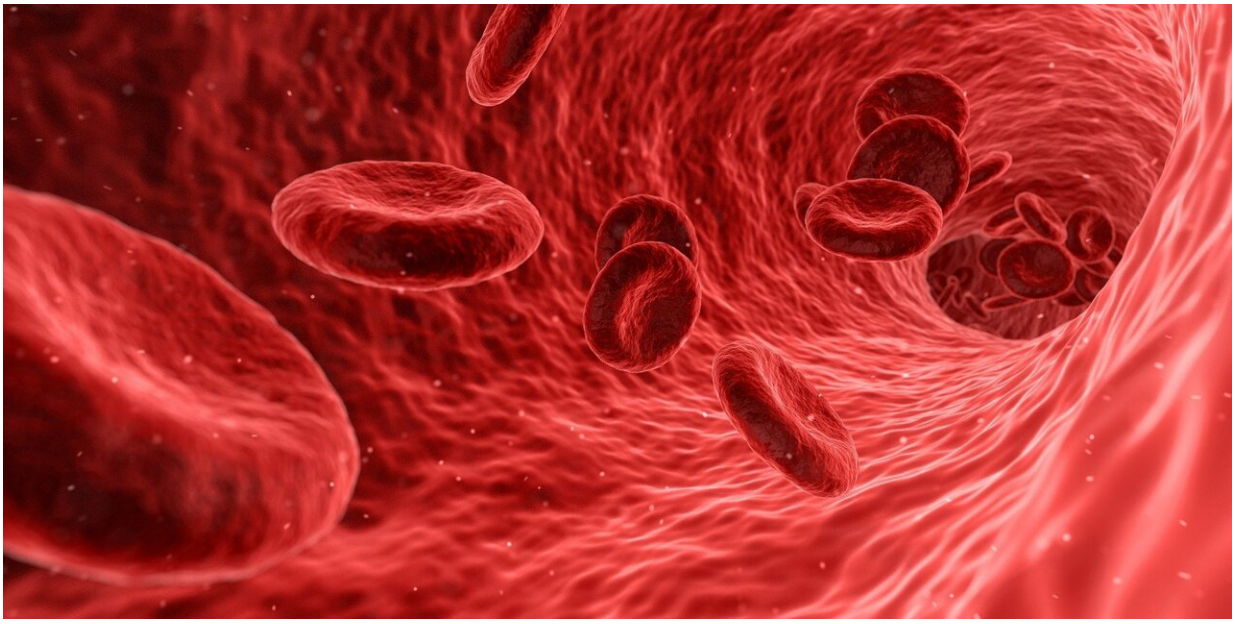
Drug found to be effective in difficult-to-treat autoimmune blood disorder

December 10 2022



Credit: CC0 Public Domain

Patients taking efgartigimod, a drug being studied for use to treat chronic primary immune thrombocytopenia (ITP), exhibited a significantly greater improvement in platelet counts which are essential to clotting and stopping bleeding, compared to those taking a placebo, according to results reported from the ADVANCE IV clinical trial, which was conducted globally, including at Georgetown University Medical Center.

People with ITP have a type of autoantibody (antibodies directed against a person's own proteins) called immunoglobulin G (IgG) that increases the clearance of platelets from the circulation and can also reduce platelet production. ITP can be very difficult to treat, especially in patients who have not responded well to previous ITP therapies.

The study findings were presented in a plenary session of the annual meeting of the American Society of Hematology in New Orleans on December 11, 2022, by Catherine Broome, M.D., associate professor of medicine at Georgetown and principal investigator in the international ADVANCE IV study.

"The results of the ADVANCE IV study provide an important answer regarding the potential benefits of efgartigimod as a treatment for ITP. There remains significant unmet need in treating ITP," says Broome. "ITP is also associated with debilitating fatigue and can have significant impacts on mental health, including anxiety, fear and depression, which is why it's been so essential to find additional therapies to treat the disease."

The annual rate of newly diagnosed cases of ITP in the U.S. is estimated to be about 3.3 new cases per 100,000 people in the general population. Between adolescence and 60 years of age, ITP is more common in females.

Efgartigimod has a novel mechanism of action. It lowers IgG levels while not affecting important immune-system components such as lymphocytes, IgG production, or the body's innate immune system.

ADVANCE IV is a phase III, double-blinded clinical trial that enrolled 131 patients in North America, Europe and Japan. The participants were randomly assigned to receive either efgartigimod or a placebo for a total of 24 weeks. All patients in the trial had low platelet counts and had at

least one ITP treatment prior to being randomly assigned in the trial; two-thirds of the enrollees had received three or more prior ITP therapies.

The drug, sold under the brand name Vyvgart, has only been approved for treatment of a form of myasthenia gravis, a condition that is caused by autoantibodies and results in broken communications between nerves and muscles.

In the ADVANCE IV trial, patients with chronic ITP who received efgartigimod compared to placebo achieved a significant improvement in sustained platelet response (21.8% vs. 5%, respectively) during at least four of the last six scheduled trial visits, with about 50% of those who responded to the drug seeing doubled platelet counts.

Response to the drug was seen in all types of patients regardless of age, disease severity, time since diagnosis, prior ITP treatment or use of other medications. The most commonly reported side effects of the drug included bruising, headache, blood in the urine and rash-like symptoms on the skin related to bleeding. No serious side effects related to treatment were reported.

"Our hope is that as more therapies are available to patients with ITP, fewer patients will experience bleeding events and fatigue, leading to an overall increase in their quality of life," says Broome.

The drug was administered intravenously in this trial. There is a concurrent trial that administers the drug subcutaneously, or just under the skin, to see if delivery that way is comparable to the intravenous administration. Results of the subcutaneous study are expected in the second half of 2023.

"Our next step is already underway," says Broome, who treats patients at MedStar Georgetown University Hospital. "ADVANCE-plus is an open-

label extension of this trial, that will provide data regarding long term effectiveness and safety of efgartigimod by observing participants for up to 60 weeks compared to the 24 weeks we're reporting on now."

More information: Conference: www.hematology.org/meetings/annual-meeting#

Provided by Georgetown University Medical Center