

Platelet drug shows clinical benefits for severe, unresponsive aplastic anemia

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Eltrombopag, a drug that was designed to stimulate production of platelets from the bone marrow and thereby improve blood clotting, can raise blood cell levels in some people with severe aplastic anemia who have failed all standard therapies.

About one-third of aplastic anemia cases do not respond to standard therapy, a combination of immune-suppressing drugs. Although [bone marrow stem cell transplantation](#) is an option for some, patients without a matched donor have few treatment options. The findings of this new clinical study, carried out by the National Heart, Lung, and Blood Institute (NHLBI) of the National Institutes of Health, suggest eltrombopag could be a second-line [therapeutic option](#) for them.

"Eltrombopag and Improved Hematopoiesis in Refractory Aplastic Anemia," will be published online July 5 in the [New England Journal of Medicine](#).

Aplastic anemia is a rare blood disorder, with about 600 new cases in the U.S. each year. Aplastic anemia results from the destruction of bone marrow stem cells, which mature into [red blood cells](#) that carry oxygen, [white blood cells](#) that fight infection, and platelets that prevent excess bleeding. Symptoms of the disorder include fatigue, frequent infections, and hemorrhaging. In severe cases unresponsive to treatment, death can occur.

Eleven of 25 participants enrolled in this phase 2 study showed improved

production of at least one type of blood cell (red blood cell, white blood cell, or platelet) after 12 weeks of oral eltrombopag therapy. Among the seven volunteers who continued taking the pills long-term (8-32 months), six eventually showed an improvement in all three types of blood cells, and were able to maintain safe blood counts without needing red blood cell or platelet transfusions. Overall the drug was well tolerated, with few side effects.

The research team in the NHLBI Hematology Branch tested eltrombopag because this drug had previously been shown to boost platelet levels in both healthy people and people with reduced platelets due to hepatitis C infection or immune thrombocytopenia, blood disorders that like aplastic anemia result in low platelet counts and increased risk of bleeding.

The encouraging finding in this study was improvement in red blood cell and white [blood](#) cell counts in some aplastic anemia patients, suggesting that the drug can stimulate bone marrow stem cells and perhaps have wider utility than initially predicted.

More information: Learn more about this study at: clinicaltrials.gov/ct2/show/NCT00922883
Learn more about aplastic anemia at: [www.nhlbi.nih.gov/health/health-topics/aplastic/](https://www.nhlbi.nih.gov/health/health-topics/topics/aplastic/)

Provided by NIH/National Heart, Lung and Blood Institute

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