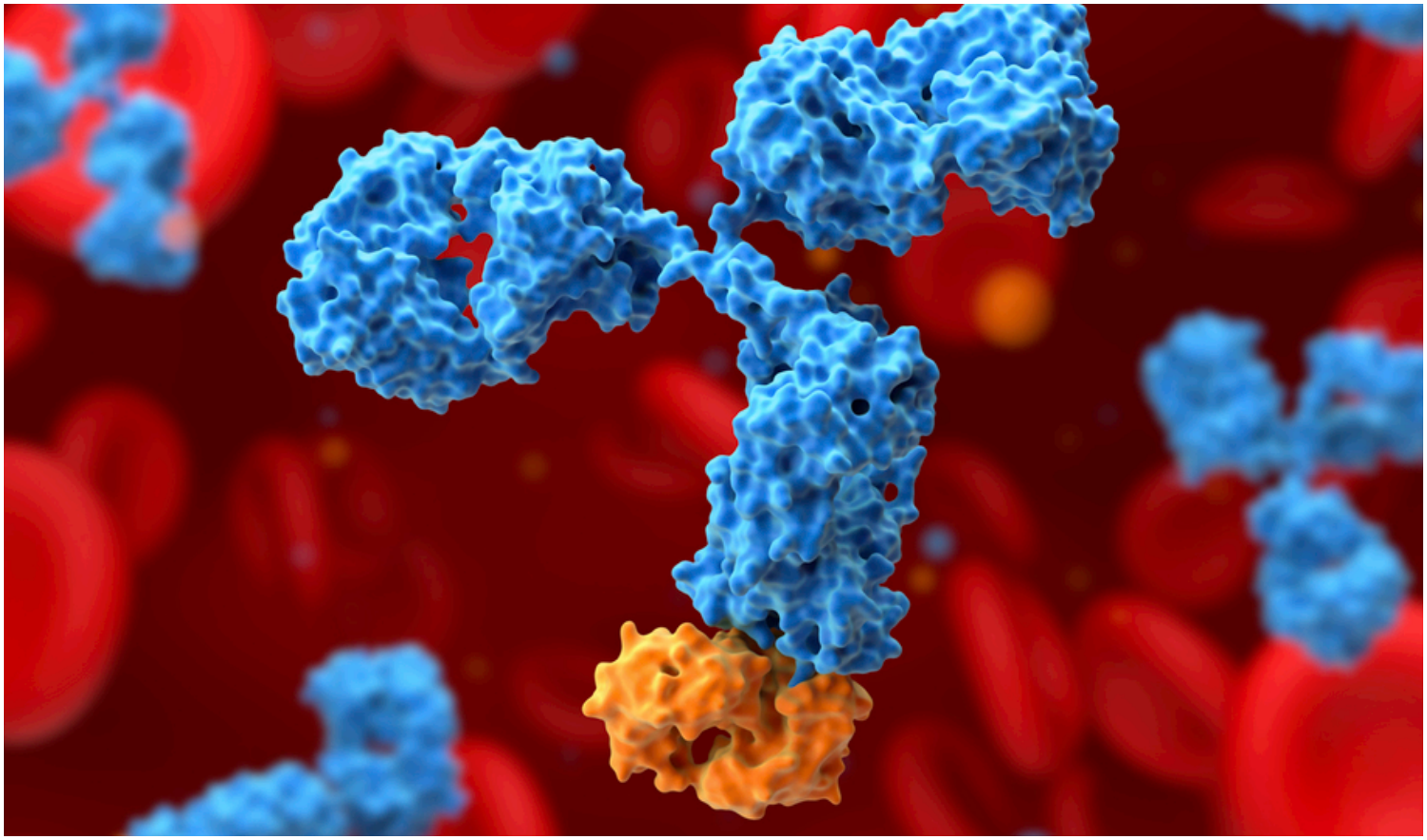
Penn Medicine

[/]

News and views

Improving treatment for an autoimmune bleeding condition



Autoimmune diseases

[/News/Search?pageTypeId=97a5141a-e62c-4dde-8fc0-160f2571b56a&articleTopics=96fbcd64-9d49-4498-9c4

|

Blood disorders

[/News/Search?pageTypeId=97a5141a-e62c-4dde-8fc0-160f2571b56a&articleTopics=4065bddd-bb3e-4902-ae

|

Clinical research

[/News/Search?pageTypeId=97a5141a-e62c-4dde-8fc0-160f2571b56a&articleTopics=b96cbe01-7f39-4db8-b75

|

Immunotherapy

[/News/Search?pageTypeId=97a5141a-e62c-4dde-8fc0-160f2571b56a&articleTopics=abf1959a-e00c-48ab-a98f

Improving treatment for an autoimmune bleeding condition

An investigational monoclonal antibody may provide extended relief for immune thrombocytopenia.

Meagan Raeke | december 7, 2025

More than half of patients in a Phase III clinical trial who received a limited course of the experimental monoclonal antibody ianalumab for primary immune thrombocytopenia (ITP), an autoimmune disorder that can cause life-threatening bleeding, were able to maintain safe platelet counts without serious bleeding episodes for at least one year. The results were published today in the *New England Journal of Medicine*

[http://www.nejm.org/doi/full/10.1056/NEJMoa2515168]

, by researchers from the Perelman School of Medicine at the University of Pennsylvania, and presented by collaborators at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition

[https://www.hematology.org/meetings/annual-meeting]

Orlando, Florida (LBA-2

[https://submit.hematology.org/program/presentation/681669]

).

ITP is an autoimmune condition where the body's immune cells mistakenly attack platelets, the blood cells responsible for clotting. It affects about

50,000 people

[https://pdsa.org/adults]

in the U.S. and can be diagnosed at any age. ITP is associated with abnormal bleeding from the skin and mucous membranes—including nosebleeds, gum bleeding, and/or heavy menstruation—that can be severe when platelet counts are particularly low. ITP also contributes to easy bruising and fatigue.

Some patients with ITP do not require treatment, but for those with low platelet counts or repeated or severe bleeding, initial treatment involves steroids, which work well for some patients. However, for patients who continue to have bleeding issues or low platelet counts with—or after tapering off—steroids, another form of treatment is required. While there are currently three FDA-approved second-line therapies for ITP, they all generally require treatment for life, either in the form of a daily pill or weekly injections, which come with their own side effects and costs.

“As a hematologist, I’m glad that we have effective therapies for ITP, but they’re not necessarily ideal for chronic disease management or long-term quality of life,” said lead author Adam Cuker, MD, MS

[/providers/adam-cuker]

, section chief for Hematology, and clinical director of the Penn Blood Disorders Center

[/Specialties/Hematology/Bleeding-disorders]

. “This study shows that prolonged, durable responses to ITP treatment, without the need for ongoing therapy, are possible—and that’s a huge advantage for patients.”

Stable platelets and successful off ramping of treatment

The double-blind, multicenter clinical trial (called the VAYHIT2 study

[https://clinicaltrials.gov/study/NCT05653219#study-overview]

) randomized 152 adult patients with ITP to three arms: a higher-dose of ianalumab (50 patients), a lower-dose of ianalumab (51 patients), or a placebo (51 patients). Ianalumab works by targeting the B-cell-activating factor (BAFF) receptor, resulting in a depletion of autoreactive B cells that are responsible for the anti-platelet antibodies that cause ITP. Patients were eligible for the study if they had already experienced a relapse after steroids or if their ITP did not respond to treatment with steroids. Ianalumab was given intravenously once a month for four months, and because it takes time to start working, all patients also received eltrombopag, one of the pills currently approved for second-line treatment. Eltrombopag is normally taken indefinitely but was intended to be tapered off and stopped for this study.

The study measured "time to treatment failure," defined as a low platelet count, the need for additional ITP therapy, inability to taper or discontinue eltrombopag, or death. The estimated probability of avoiding treatment failure at 12 months was 54.2 percent in the high-dose arm and 50.5 percent in the low-dose arm, versus only 30 percent of patients in the placebo arm. Additionally, when platelet counts were measured at six months (two months after the last dose of ianalumab), 62 percent of patients in the high-dose treatment arm had stable platelet counts, versus only 39.2 percent of patients in the placebo arm.

'A new era of hope'

Additional clinical trials for ianalumab are ongoing, including in studies for other autoimmune conditions, and it is not yet FDA-approved for patients. The researchers will continue to follow the patients from this study to track long-term response to treatment.

“We’re looking forward to seeing if the treatment-free responses in this study extend out even further,” Cuker said. “Improving the long-term reality of living with ITP is not something we’ve been able to think about before. The goal has always been to improve platelet counts or reduce the risk of bleeding, but this research is ushering in a new era of hope for patients with ITP.”

The study was funded by Novartis.

Media contact

Meagan Raeke

C: 267-693-6224

[tel:267-693-6224]

Meagan.Raeke@pennmedicine.upenn.edu

[mailto:Meagan.Raeke@pennmedicine.upenn.edu]

For Media Queries & Requests (24/7): 215-662-2560

[tel:215-662-2560]

Follow us

[https://x... [https://w... [https://... [https://... [https://www.linkedin.com/company/university-

Subscribe

Subscribe to Penn Medicine newsletters and publications for the latest developments.

Subscribe [/News/Subscribe]

Learn more about Penn Medicine

About us

[/About]

Pioneering the future of medicine

[/About/Pioneering-the-future-of-medicine]

Transforming patient care

[/About/Transforming-patient-care]

Supporting our communities

[/Supporting-our-communities]

For healthcare professionals

Refer a patient

[/Physician-resources/Refer-patient]

PhysicianLink login

[https://secure3.pennmedicine.org/EpicLink/common/epic_login.asp]

Physician resources

[/Physician-resources]

For international patients

Global medicine

[https://global.pennmedicine.org/en]

Ways to help

Giving

[/Giving]

Volunteering

[/Volunteering]

Organ donation

[/Specialties/Transplant/Living-organ-d

Patient & family advisors

[/Patient-resources/Information-for-pat

Research & innovations

Research

[/Research]

Office of Clinical Research

[https://www.med.upenn.edu/clinicalres

For employees

Employee resources

[/Employee-resources]

[https://www.linkedin.com/company/universit

Contact us [/Contact-us]

HIPAA & privacy [/Hipaa-and-privacy]

Terms of use [/Patient-resources/Policies/Terms-of-use]

Legal disclaimer [/Patient-resources/Policies/Legal-disclaimer]

Price transparency [/Patient-resources/Policies/Pricing-transparency]