RAIN
Rare Anaemias
International Network

EHA 2026 Research Highlights

What the latest research means for people living with rare anaemias

June 2026

Powered by the Thalassaemia International Federation (TIF)

EHA 2026: Hope Through Research

Every year, researchers, healthcare professionals and patient advocates meet at the European Hematology Association (EHA) Congress to share the latest discoveries in blood disorders. The 2026 meeting highlighted **important progress across many rare anaemias**, including new medicines, advances in gene and mRNA therapies, and a stronger focus on personalised care and quality of life.

Across the abstracts, the main results were encouraging: several treatments increased haemoglobin, reduced transfusion needs, improved fatigue, and were generally well tolerated. Many studies also showed that patients value treatments that are easier to take, require fewer hospital visits, and fit better into daily life.

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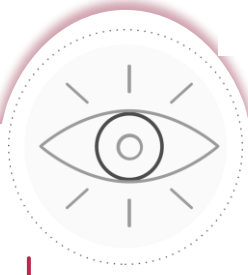

Vision

To empower and strengthen patient advocacy groups supporting rare anaemias by establishing a global network for the dissemination and exchange of knowledge and best practice.
To establish a global voice in rare anaemias through community collaborations.

Mission

RAIN aspires to improve the health and quality of life of people with rare anaemias by:
Implementing and/or establishing national policies specific to rare anaemias
Ensuring equal access to diagnosis and treatment
Initiating and supporting research by bringing the patient perspective to the drug development process
Rendering novel therapies accessible to patients

Paroxysmal Nocturnal Haemoglobinuria (PNH)

PNH is caused by an acquired mutation in blood-forming stem cells that makes red blood cells vulnerable to destruction by the complement system, a part of the immune system. This leads to haemolysis, which means breakdown of red blood cells.

What's new?

- Studies on shared decision-making showed that when patients were more involved in choosing treatment, care plans were better matched to what mattered most to them, such as fewer infusions and less disruption to daily life.
- New research is improving understanding of how the bone marrow and immune system interact in PNH, which may help explain why some patients respond differently to treatment.
- Several newer medicines showed clear clinical benefit:
  - **Ravulizumab** maintained complement control with less frequent dosing than older treatments.
  - **Danicopan** improved haemoglobin in patients who still had anaemia despite standard complement blockade.
  - **Pegcetacoplan** improved haemoglobin and reduced transfusion needs in patients with ongoing haemolysis.
  - **Iptacopan**, an oral medicine, improved haemoglobin and lowered the need for transfusions in many patients.
- Oral medicines and treatments with less frequent dosing were attractive because they may reduce the burden of long-term therapy.
- Some patients remained anaemic despite treatment with **eculizumab**, showing that one treatment does not fit everyone.

What this means for patients

Treatment options for PNH are expanding quickly. The studies suggest that newer therapies can improve anaemia, reduce transfusions and make treatment easier to live with. For many patients, the future may mean fewer hospital visits and more personalised treatment choices.

Fanconi Anaemia

Fanconi anaemia is an inherited bone marrow failure syndrome caused by defects in DNA repair pathways. Because the DNA damage is not repaired properly, blood stem cells can fail over time.

What's new?

- Researchers presented **early gene therapy results** in which a working copy of the faulty gene was inserted into the patient's own blood stem cells.
- In these early studies, treated stem cells were able to engraft, meaning they survived and started making blood cells.
- Some participants showed improving blood counts and reduced need for supportive care, although the studies were still small and early.

What this means for patients

Gene therapy is still experimental, but these early results are important because they suggest it may one day treat the underlying cause of Fanconi anaemia rather than only managing symptoms and complications.

🦴 Aplastic Anaemia

Aplastic anaemia occurs when the bone marrow fails to produce enough blood cells, often because of immune-mediated damage to blood-forming stem cells. This can cause anaemia, infections and bleeding.

What's new?

- Researchers, across multiple studies that included various aplastic anaemia forms (e.g. hepatitis-associated, relapsed, failed-to-respond, familial, and moderate) identified immune and genetic changes that may help explain why aplastic anaemia develops and why some patients respond better than others.
- Oral treatment combinations, especially **eltrombopag plus cyclosporine**, gave encouraging results. In the studies presented, many patients had improved blood counts, and some became less dependent on transfusions.
- Studies also highlighted inherited and familial forms of aplastic anaemia, which are important to recognise because they may need different management.
- Genetic studies using **next-generation sequencing (NGS)** demonstrated that acquired genetic (somatic) mutations are common in AA and can influence how the disease progresses, including the risk of developing **myelodysplastic syndrome (MDS)** or **acute myeloid leukaemia (AML)**. These findings may help doctors better predict outcomes and select the most appropriate treatment before transplantation.

🔍 What this means for patients

Aplastic anaemia is becoming better understood at both the **immune** and **genetic** level. New oral treatment combinations such as **eltrombopag plus cyclosporine** are showing promising results and may allow more patients to receive effective treatment without prolonged hospital stays. At the same time, advances in genetic testing and immune profiling are moving care towards **more personalised treatment**, helping doctors identify which patients are most likely to benefit from specific therapies or require closer monitoring. Despite this progress, patients whose disease relapses or does not respond to current treatments still have limited options, highlighting the ongoing need for new therapies and continued research.

Pyruvate Kinase Deficiency (PKD)

PKD is an inherited disorder in which red blood cells cannot produce energy efficiently and are destroyed prematurely. This causes chronic haemolytic anaemia.

What's new?

- Real-world studies confirmed that **mitapivat** increased haemoglobin levels in many adults with PKD.
- The studies also showed fewer transfusions in patients who had previously needed regular blood support.
- Some patients reported better energy levels and improved day-to-day functioning.

🔍 What this means for patients

For some people with PKD, mitapivat can reduce anaemia, lower transfusion needs and improve quality of life. These results support its role as an important treatment option for adults with this condition.

Autoimmune Haemolytic Anaemia (AIHA)

In AIHA, the immune system produces antibodies that mistakenly target and destroy red blood cells. This causes haemolysis and anaemia.

What's new?

- **Nipocalimab** showed rapid improvements in haemoglobin and fatigue in the studies presented, and it was generally well tolerated.
- The abstracts also showed that many patients still do not achieve long-term disease control with current treatments, especially if they relapse after steroids or other immunosuppressive therapy.
- These findings support the need for treatments that work more consistently and with fewer side effects.

🔍 What this means for patients

New treatments may offer better control of anaemia and tiredness, with fewer steroid-related problems. This is especially important because long-term steroid use can cause significant side effects.

Warm Autoimmune Haemolytic Anaemia (wAIHA)

A rare autoimmune disease where the immune system produces IgG antibodies that mistakenly attach to red blood cells. These antibody-coated red blood cells are then destroyed, mainly in the spleen, leading to haemolytic anaemia.

What's new?

- A large real-world study from Sweden showed that **oral corticosteroids** remain the standard first treatment for most people with wAIHA. **Rituximab** is usually added only for patients with more severe disease.
- Despite current treatment recommendations, many patients either **did not respond adequately** to first-line treatment or **relapsed** after an initial improvement. As a result, many required additional ("rescue") treatments because their anaemia remained poorly controlled.
- These findings highlight that current first-line treatments do not provide **lasting disease control** for many patients, emphasising the need for new treatment options.
- Researchers from Canada also presented one of the largest studies to estimate how common wAIHA is in the general population. They estimated that approximately **2–3 people per 100,000 develop wAIHA each year**, while around **12–16 people per 100,000 are living with the disease** at any one time.
- The study confirmed that wAIHA is **more common in women** and becomes increasingly common with **older age**.
- Long-term follow-up over six years showed that many patients continued to have persistent anaemia, demonstrating that wAIHA often remains an ongoing chronic condition despite treatment.

What this means for patients

Current treatments can be effective, but many people experience relapses or continue to have active disease reinforcing the need for new therapies that provide more durable disease control, reduce the need for repeated steroid treatment and blood transfusions, and improve long-term quality of life.

🩸 Erythrocyte Membrane Disorders and Congenital Dyserythropoietic Anaemia (CDA)

These inherited disorders affect either the structure of red blood cells or their production within the bone marrow. They can cause chronic anaemia, jaundice, gallstones or iron overload.

What's new?

- **Mitapivat** improved haemoglobin in some inherited red cell disorders and also reduced iron overload markers in certain patients.
- Studies on fertility and pregnancy were reassuring overall. Most pregnancies had good outcomes, but women with **CDA** and **PKD** may still need closer monitoring because anaemia and iron overload can affect pregnancy.
- New laboratory models of **CDA type II** may help researchers test future treatments more quickly and accurately.

🔍 What this means for patients

These studies show that researchers are beginning to understand these rare disorders in more detail. That could lead to better treatment choices, safer pregnancy planning and more targeted therapies in the future.

❄ Cold Agglutinin Disease (CAD)

CAD is an autoimmune haemolytic anaemia in which antibodies activate the complement system and cause red blood cell destruction, especially at lower temperatures.

What's new?

- Researchers gained new insights into the molecular mechanisms behind CAD, helping explain how the disease starts and why it persists.
- Long-term studies showed that **riliprubart** continued to have a favourable safety profile, with no major new safety concerns reported.
- These findings support the idea that complement-targeted treatment can be effective and manageable over time.

🔍 What this means for patients

A better understanding of CAD may help doctors choose treatments more precisely and improve long-term control of symptoms such as fatigue, cold-induced problems and anaemia.

Diamond-Blackfan Anaemia Syndrome (DBAS)

DBAS is an inherited bone marrow failure syndrome caused by abnormalities in proteins involved in ribosome production. Ribosomes are needed to make proteins, so problems here can affect red blood cell production.

What's new?

- Researchers are investigating **mRNA-based therapies**, which aim to replace missing proteins without changing a person's DNA.
- Early laboratory work suggests that this approach could restore protein production in affected cells.
- Studies also reinforced the importance of genetic testing, especially in adults with unusual or late-presenting disease.

🔍 What this means for patients

mRNA therapy is still at an early stage, but it could become a new way to treat inherited bone marrow failure disorders. Genetic testing remains important because it can confirm the diagnosis and guide care.

Primary Chronic Neutropenia

Primary chronic neutropenia is characterised by persistently low neutrophil counts, increasing the risk of recurrent and severe infections. Neutrophils are a type of white blood cell that help fight bacteria.

What's new?

- An international phase 3 trial is evaluating **mavorixafor**, an oral medicine designed to increase neutrophil counts.
- Early results from the trial programme suggest that the drug can raise neutrophil levels and may reduce infection risk in some patients.
- Because it is taken by mouth, it may be easier to use than injectable treatments.

What this means for patients

If the trial confirms benefit, mavorixafor could become a simpler long-term treatment option that helps reduce infections and improves daily life.

Sources

Plenary Abstracts Session & Oral Presentations

https://doi.org/10.1002/hem3.70419

Poster Session

https://doi.org/10.1002/hem3.70420

Publication Only Abstracts

https://doi.org/10.1002/hem3.70421