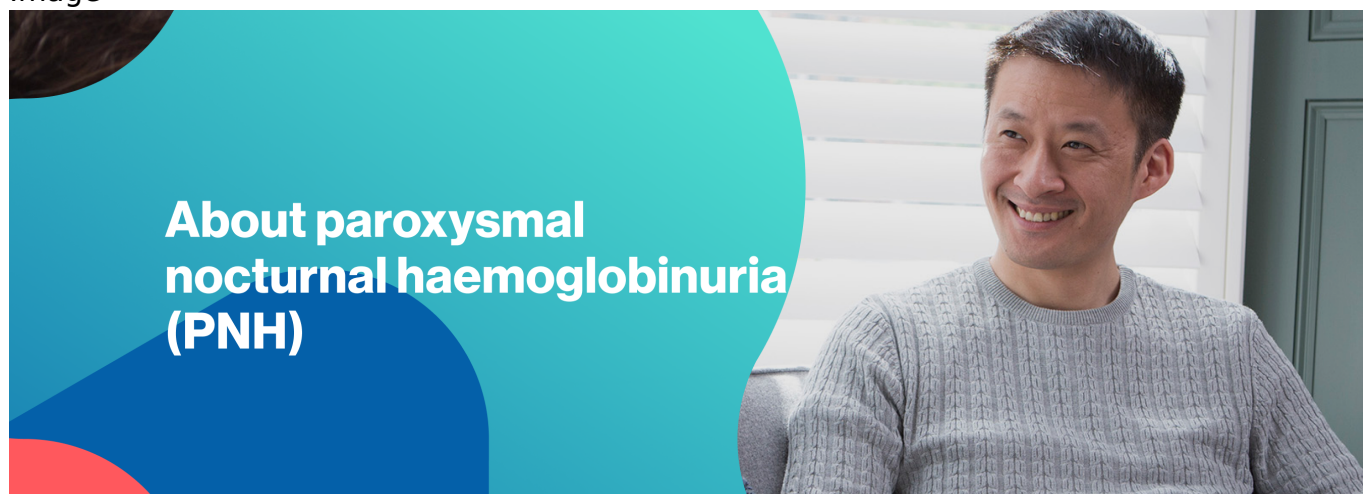


FABHALTA - About PNH - MOP

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Image



**About paroxysmal  
nocturnal  
haemoglobinuria  
(PNH)**

This page is intended for UK patients who have been prescribed iptacopan for paroxysmal nocturnal haemoglobinuria.

The information on this website does not replace your patient information leaflet. Please always refer to your patient information leaflet for more information. Fabhalta Connected – Stronger Together is a patient support programme organised and funded by Novartis.

# About paroxysmal nocturnal haemoglobinuria (PNH)

## What causes PNH?

Paroxysmal nocturnal haemoglobinuria (PNH) is a rare blood disorder that happens when part of your immune system attacks and destroys its own red blood cells (RBCs) in a process called haemolysis. RBCs contain a protein called haemoglobin that carries oxygen around your body.<sup>1</sup> As RBCs are broken down, the level of haemoglobin goes down, causing symptoms including anaemia.<sup>2-5</sup>

## What happens in PNH

1. **Normal red blood cells (RBCs) have a protective shield** of proteins to defend against any attack from the complement system (part of your immune system).<sup>2,4,5</sup>
2. In your body, **RBCs are missing key proteins that create this protective shield.** This leaves them vulnerable to attack.<sup>2-5</sup>
3. The complement system is overactive in PNH and **forms a membrane attack complex (MAC) that punctures holes into unprotected RBCs**, causing them to fall apart.<sup>3,5</sup>
4. The destruction of RBCs is known as **haemolysis**. It is the reason why you may experience symptoms such as **tiredness, dark or red urine** (from haemoglobin in the blood), **shortness of breath** and **pain**. This overactivity varies from person to person and so can the number of missing 'protective' proteins.<sup>2,4,5</sup>

Image



### 1. Normal RBCs

Have a protective shield

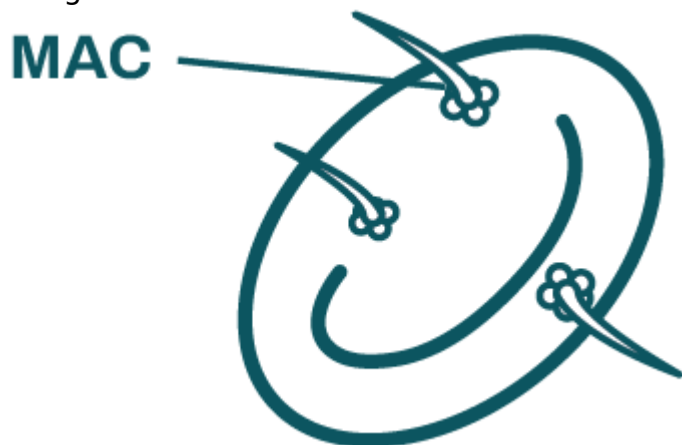
Image



### 2. RBCs in PNH

Lack key proteins needed to protect them from damage

Image



### 3. Complement attack

The complement system's MAC punctures holes into unprotected RBCs

Image



#### **4. PNH RBC destruction (haemolysis)**

PNH RBCs fall apart and the contents released in the liquid part of the blood known as plasma

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## **What are the common signs and symptoms of PNH?**

Many people with PNH experience the following symptoms:<sup>5-7</sup>

- Anaemia, leading to fatigue, brain fog, shortness of breath and easy bruising
- Dark-coloured urine
- Stomach pain
- Difficulty swallowing
- Erectile dysfunction

PNH can lead to serious complications, including bone marrow failure, blood clots, and kidney damage or failure.<sup>5,8</sup> Not everyone with PNH will have the same symptoms, and your symptoms will vary. You may have different symptoms at different times, and only when you have a severe episode of haemolysis. Alternatively, you may experience PNH symptoms all the time.<sup>6-8</sup>

**Click to access the PNH symptom tracking tool**

[Access](#)

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# What is haemolysis?

Haemolysis is the breakdown of red blood cells.<sup>6</sup> The bone marrow produces different types of blood cells including white blood cells, platelets, and red blood cells. In PNH, a change in one or more genes (known as a mutation) occurs and the bone marrow produces blood cells that may be missing certain protective proteins. Without these proteins blood cells aren't protected in the same way against "complement activation", a part of the body's normal immune response that fights infections. As a result, the body's immune system attacks and destroys red blood cells, leading to haemolysis.<sup>4,9</sup>

## What is the complement system?

Your body's natural immune system defends your body against disease and infection. The complement system forms part of this, and is active in the process that leads to haemolysis and anaemia:<sup>4,9,10</sup>

- The complement system is made up of more than 40 different proteins. As well as fighting disease, it identifies damaged cells and breaks them down<sup>11,12</sup>
- When the complement system identifies red blood cells that are missing their specific protective protein, it mistakes these cells for abnormal cells and becomes overactive, resulting in the breakdown of the red blood cells, causing haemolysis<sup>4,9,10</sup>
- Specific proteins in the complement system are involved in this response - C3, C5, and Factor B. These help to continue the overactive immune response that ultimately causes you to experience symptoms of anaemia<sup>4,10,11</sup>

## How is anaemia managed?

Because anaemia can take a toll on your life and cause complications, it's important to treat it. One way to manage anaemia is with blood transfusions. Usually, you can have your transfusions in a hospital outpatient clinic. You can expect it to take about two hours to transfuse one unit of blood into your body.<sup>13,14</sup>

You'll typically feel better after a transfusion, as the symptoms caused by anaemia are eased. But this relief may be temporary and you'll need another transfusion if the anaemia comes back.<sup>15</sup>

## Resources & FAQs

Here you can download tools that can help you manage your treatment and condition, as well as find answers to questions you may have about iptacopan or PNH. There is also information on local support groups and useful websites.

[Sign up](#)

## References

1. Mayo Clinic. Anemia. Available at: <https://www.mayoclinic.org/diseases-conditions/anemia/symptoms-causes/syc-20351360>. [Accessed October 2024].
2. PNH Support. What is PNH? Available at: <https://pnhuk.org/what-is-pnh/>. [Accessed October 2024].
3. Risitano AM, et al. EHA 2023, 8–11 June; Frankfurt, Germany. Oral S182.
4. Dingli D, et al. *Ann Hematol* 2022;101(2):251–263.
5. Brodsky RA. *Blood* 2014;124:2804–2811.
6. Cleveland Clinic. Hemolysis. Available at: <https://my.clevelandclinic.org/health/diseases/24108-hemolysis>. [Accessed October 2024].
7. PNH Support. Anaemia. Available at: <https://pnhuk.org/what-is-pnh/signs-and-symptoms/>. [Accessed October 2024].
8. Bektas M, et al. *J Manag Care Spec Pharm* 2020;26(suppl12b):S3–S8.
9. Hillmen P, et al. *Br J Haematol* 2013;162:62–73.
10. Merle NS, et al. *Front Immunol* 2015;6:262.
11. Mastellos DC, et al. *Expert Rev Hematol* 2014;7:583–598.
12. Bardhan M, et al. Physiology, Complement Cascade. StatPearls. 2023. Available at: <https://www.ncbi.nlm.nih.gov/books/NBK551511/>. [Accessed October 2024].
13. NORD. Anemia, Hemolytic, Acquired Autoimmune. Available at: <https://rarediseases.org/rare-diseases/anemia-hemolytic-acquired-autoimmune/>. [Accessed October 2024].
14. PNH Support. Current Treatments. Available at: <https://pnhuk.org/what-is-pnh/current-treatments/>. [Accessed October 2024].

### **Reporting side-effects**

If you get side-effects with any medication you are taking, talk to your doctor, pharmacist or nurse. This includes any possible side-effects not listed in the information leaflet that comes in the pack. ▼ The medicine referred to in this material is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side-effects you may get. Please see [www.mhra.gov.uk/yellowcard](http://www.mhra.gov.uk/yellowcard). for instructions on how to report side-effects.

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By reporting side-effects you can help provide more information on the safety of your medication.

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