



# My Voice My PNH Care

This booklet is designed to help support you in making the most of your care.



Being diagnosed with PNH can come as a shock, but knowing the facts can make everyday life with the disease a little bit easier.

Read on to find out more...

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# Understanding PNH

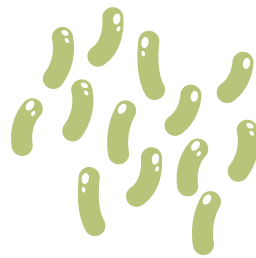
## What is PNH?

Paroxysmal Nocturnal Haemoglobinuria, or PNH, is a bone marrow disease that is caused when the cells responsible for making red blood cells (which help to carry oxygen around the body) mutate and produce defective blood cells.<sup>1</sup> This triggers the immune system – a sophisticated defence network that keeps the body safe from dangers like disease and infection – to attack and destroy these defective red blood cells, a process called haemolysis.<sup>2</sup>

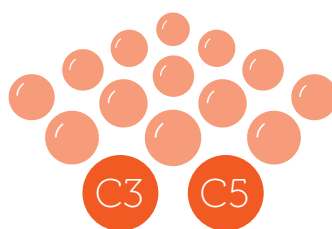
PNH is a rare disease, but it can affect anyone of any age, race or gender. No one is born with PNH; it is known as an 'acquired disease', which means it cannot be inherited and it is not contagious.<sup>3</sup>

## More on haemolysis

Haemolysis is the process by which various red blood cells are 'broken apart' by different factors, such as the immune system, and it occurs after a cascade of activity has been started in the 'complement system'.<sup>3</sup>



The complement system helps destroy dangerous toxins, viruses or bacteria, and gets rid of damaged or dying cells<sup>4</sup>



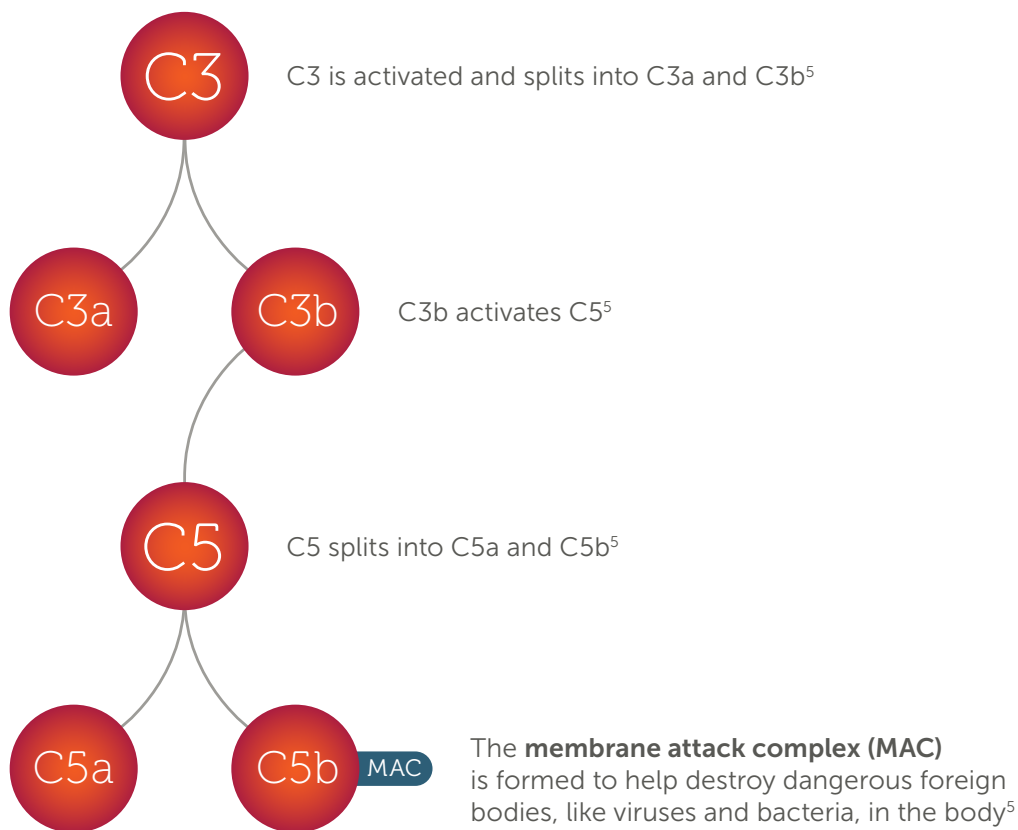
There is an army of more than 60 different **proteins** that play a part in the complement system. Two key proteins are known as **C3** and **C5**<sup>4</sup>



Like dominoes, C3 and C5 proteins are activated in a specific order as your immune system detects viruses, bacteria or foreign structures (toxins)<sup>4</sup>

## The Complement System

The immune system is a sophisticated defence network that keeps the body safe from dangers like disease and infection. One very complex component in this network is called 'the complement system'.<sup>4</sup>



## There are 2 types of haemolysis: intravascular and extravascular

Both types may occur in your body at the same time and cause the PNH symptoms you experience<sup>1,2</sup>



### INTRAVASCULAR HAEMOLYSIS

IVH occurs when red blood cells are destroyed inside the blood vessels – the channels through which blood is distributed to body tissues<sup>1,2</sup>



### EXTRAVASCULAR HAEMOLYSIS

EVH occurs when red blood cells are destroyed in the liver and spleen<sup>1,2</sup>

## Diagnosing and monitoring PNH

Your doctor will consider all your signs and symptoms when diagnosing and then monitoring your PNH. They will also look at a variety of test results, such as blood tests, as these can provide valuable clues about what is happening in your body.<sup>1</sup> It is important that you understand some of the terms you will hear:

### Haemoglobin (Hb):

a protein that carries oxygen and carbon dioxide in red blood cells. PNH is characterised by a low number of red blood cells and low haemoglobin levels<sup>1,6</sup>

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### Lactate Dehydrogenase (LDH):

an enzyme found in all cells. Red blood cell destruction results in the release of LDH into the blood so high LDH levels may be an indication that IVH is ongoing<sup>1,6,7</sup>

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### Reticulocytes:

immature red blood cells. High reticulocyte counts mean your body is working too hard to replace the red blood cells being destroyed by PNH<sup>1</sup>

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### Bilirubin:

a pigment produced from the breakdown of red blood cells. People with PNH may have high levels of bilirubin<sup>6</sup>

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It's always best to speak to your doctor about your test results and what they may mean for your health.

## Symptoms of PNH

PNH symptoms vary from person to person. Some people have just a few while others can be very unwell with serious, debilitating issues with serious debilitating symptoms that can impact daily life.<sup>1,7-11</sup>

These potential symptoms include:<sup>1,7-11</sup>



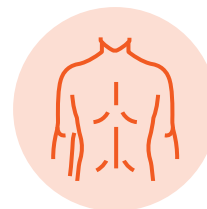
**Fatigue** – tiredness



**Difficulty concentrating**  
– 'brain fog'



**Anaemia**



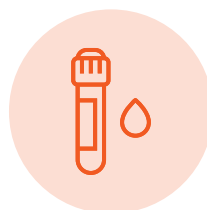
**Back and abdominal pain**



**Severe headache**



**Blood clots**



**Haemoglobinuria**  
– blood in urine



**Involuntary smooth muscle contractions in internal organs** can cause further complications such as:

- **Dyspnoea** – difficulty breathing
- **Dysphagia** – difficulty swallowing
- **Erectile dysfunction**

## Anaemia

Anaemia is defined as a **lower-than-normal number of red blood cells**. Low red blood cell counts can occur due to a *reduced production* of red blood cells, resulting from poor bone marrow function, or due to an *increased destruction* of red blood cells (haemolysis).<sup>7</sup>

One of the primary characteristics of classical PNH is **haemolytic anaemia**.<sup>1,7</sup> A reduced number of red blood cells means that the blood's capacity to carry oxygen to the body's tissues is also reduced, resulting in symptoms such as **fatigue, weakness, dizziness and shortness of breath**.<sup>12</sup>

## Fatigue

Up to **96%** of people living with classical PNH experience fatigue.<sup>8</sup>

# Living with PNH

## Physical health & wellbeing

PNH can significantly affect your quality of life, with fatigue often cited as the most challenging symptom which has an impact on daily living.<sup>13</sup>

As PNH is a complex disease that impacts each person differently, not every lifestyle change will work for everybody. You might need to experiment with different lifestyle modifications to find out what works for you.



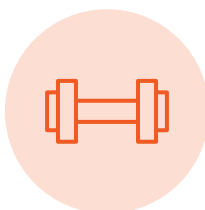
### Rest

Ensuring you get sufficient **rest** – with naps where possible – may help you to manage fatigue.<sup>13</sup>

It can be helpful to keep **regular sleeping patterns** and do some **light exercise** during the day to help you to get a better night's sleep.<sup>14</sup>

Think about **avoiding drinks containing caffeine** and **alcohol** which can interfere with the body's ability to rest.<sup>14</sup>

Worry and anxiety can sometimes impact sleep. To help you relax, you could try taking a warm **bath before bed**. It can also help to get out of bed and **go into another room**, read a book or listen to music.<sup>14</sup>



### Exercise

It can be helpful to do some **physical activity** if you feel able to.<sup>15</sup>

Physical activity can range from **gentle exercises**, such as **walking, yoga** and light housework, to more **intensive activities**, such as **cycling or running**.<sup>15</sup>

Speak to your healthcare team about what might be right for you.<sup>15</sup>



### Diet

While there is no special diet that can alleviate all your PNH symptoms, it's important to eat a **well-balanced diet** containing lots of **fruits and vegetables**.<sup>15</sup>

Your healthcare team will be able to advise you.

## Mental health & wellbeing

Mental health and wellbeing is important for a good quality of life. A PNH diagnosis is often life-changing and it's normal to experience stress or anxiety about your condition, symptoms or treatment.<sup>16</sup>

**Gentle exercise, meditation** and **journaling your experiences** may be useful for managing stress and anxiety.<sup>17,18</sup>

If your diagnosis has made you feel quite lonely or disconnected, talking or sharing your experiences with other PNH patients may help.

Support groups, whether in your community or online, may help you connect with other PNH patients, who may be experiencing the same feelings as you. PNH Global Alliance is a great source for finding these supportive organisations.

As with strategies for helping your physical wellbeing, to manage your mental health and wellbeing, it may be helpful to keep an **open mind** and to **try different approaches** until establishing what works best for you. Mind or The Mental Health Foundation are great sources for guidance.





## Work and family life

Balancing work and family life can be challenging when feeling well. Balancing these while trying to cope with the symptoms and emotions that result from PNH may be quite difficult. It's important to plan ahead and **ask for help**.

### **Rationalise!**

Think about your priorities and what can be realistically managed each day. Where possible, save energy for the things that are most important.

### **Don't do it alone!**

You might need support from family and friends to relieve some of the additional pressures caused by PNH, so do ask for help should you need it. And be very specific about what you need.

It may be stressful and anxiety-provoking to let an employer know about a chronic health condition and the need for frequent treatments.

It may be helpful to be open and honest with your employers about the challenges that your disease presents and what extra time and support you might need.

Your healthcare team should be able to act as an advocate in these situations and provide support, such as a medical letter.



## Communication is key



### Family and friends

PNH is a relatively unknown condition, which means you may have to explain it to **friends, family, neighbours and colleagues**. This may be difficult, but it will help those around you understand how best to support you.

It may be helpful to be open and honest when someone asks how you're feeling, so they can understand how the disease is impacting you.

It can also be helpful to let those around you know as your needs change, as it will help them feel connected to you.



### Healthcare professionals

**A good relationship with your healthcare team** is important for the successful management of your disease. Every appointment and conversation is important. Preparing for your appointment may take a little time, but it will help the team provide you with the best support.

## 1. Before your appointment

**Planning for your appointment** can help you get the most out of the conversations with your healthcare team. Consider writing down the questions you want to ask before your appointment, for example:

- "What do my results mean?"*
- "What further tests will be required?"*
- "What are my treatment options? How might they make me feel?"*
- "When will I be assessed again?"*

## 2. At your appointment

- **Share as much information as possible** – it's crucial that doctors and nurses are fully informed of all symptoms, even if they don't appear to be important or related to PNH. This might include information on how you are feeling physically or emotionally.

Consider:

### **How often do you feel fatigued and what is the impact?**

*Be specific about how you feel – using numbers can help (e.g., "I had to miss work 3 times last month due to fatigue")*

### **How often do you feel shortness of breath and does that stop you enjoying life?**

*(e.g., "I have had to give up my exercise class because I can't keep up with the group")*

### **What specifically are you finding challenging?**

*(e.g., "I keep having to ask my parents to look after my children because I am too tired to enjoy weekend activities")*

- Ask for guidance to support services
- Ask for clarification if you don't understand something
- Set clear next steps – leave with a full understanding about what will be happening and when

## 3. After your appointment

- Keep track** – note down how changes to your routine or treatment plan are affecting you, as well as any new topics or observations to discuss with your doctor at your next appointment
- Stay organised** – keep a record of all your appointments and results in one place so they can be easily shared with new doctors or specialists

# Symptom tracking

You may not think the minor changes in your symptoms from day to day are important, or even notice them but your signs and symptoms over time, combined with your blood results, are important for recognising changes in your health. Consider keeping a symptom tracker or diary.

## Symptom tracker

Check the answer that best describes your experience living with PNH

How many days a month do you experience:

Abdominal pain and/or chest pain

Never  1 or 2 days  3–5 days  6+ days

Leg and/or back pain

Never  1 or 2 days  3–5 days  6+ days

Trouble swallowing

Never  1 or 2 days  3–5 days  6+ days

Difficulty focusing/thinking clearly or 'brain fog'

Never  1 or 2 days  3–5 days  6+ days

How many times in the last month did you experience:

Fatigue

Never  1 or 2 times  3–5 times  6+ times

If you do experience fatigue, how would you classify the severity of the fatigue you experience?

Mild  Moderate  Severe

Headaches

Never  1 or 2 times  3–5 times  6+ times

Shortness of breath

Never  1 or 2 times  3–5 times  6+ times

Difficulty sleeping/feeling of weakness

Never  1 or 2 times  3–5 times  6+ times

Inability to keep up with your daily life due to PNH

Never  1 or 2 times  3–5 times  6+ times

Loss of appetite

Never  1 or 2 times  3–5 times  6+ times



After checking your answers, share the results with your doctor to start a conversation about your PNH experience



# Glossary

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## Anaemia

- A condition that occurs when there are not enough red blood cells to effectively carry oxygen around the body, which can cause fatigue, shortness of breath and headaches.

## C3

- A protein located in the blood that acts as a central point of the complement system, regulating its activation. In IVH, C3 tags red blood cells and activates C5, leading to MAC formation and destruction of red blood cells, and in EVH, C3 deposition causes destruction by macrophages.

## C5

- A protein located in the blood that plays an important role in inflammation and intravascular haemolysis caused by the complement system.

## Complement system

- The complement system, also referred to as the complement cascade, is an army of more than 60 different proteins that work together to get rid of dangerous cells and fight infection.

## Extravascular haemolysis (EVH)

- Destruction of red blood cells that occurs in the liver or spleen.

## Fatigue

- Extreme tiredness or exhaustion. Fatigue is one of the most common symptoms of PNH.

## Haemolysis

- The 'breaking apart' of red blood cells. It can occur when the immune system attacks these cells as though they were dangerous viruses or bacteria. When red blood cells break open, haemoglobin is released. Haemolysis causes many of the symptoms of PNH.

## Immune system

- A sophisticated defence network used to protect the body from dangers like disease and infection.

## Intravascular haemolysis (IVH)

- The destruction of red blood cells inside a blood vessel.

## Membrane attack complex (MAC)

- A structure that is formed within the chain of complement system activation. It attaches to cells which the body believes are threatening and helps destroy them.

## PNH red blood cells

- Red blood cells that are missing protective proteins, which target them for an attack by the complement system, causing the 'breaking apart' of red blood cells, a process called haemolysis.

## Proteins

- Large, complex molecules that play many important roles in the body and can be thought of as the 'workhorses' of cells. Proteins are required for the structure, function and regulation of the body's organs and tissues.

## Red blood cells

- The most common type of blood cell. Their job is to carry oxygen, using an important molecule, haemoglobin, around the body.

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# Improving life for people with PNH