Your Guide to Understanding PNH

(Paroxysmal Nocturnal Hemoglobinuria)

The Aplastic Anemia & MDS International Foundation, Inc. is an independent nonprofit organization. Our mission is to support patients, families, and caregivers coping with:

- Aplastic anemia
- MDS (myelodysplastic syndromes)
- PNH (paroxysmal nocturnal hemoglobinuria)
- Related bone marrow failure diseases

This booklet offers you information about PNH and how it is diagnosed and treated. Although the information in this booklet has undergone a thorough, independent medical review to insure its accuracy, this information is not intended to be a substitute for the advice of your doctor. You should always seek medical advice from a qualified physician.

For more information, call us at (800) 747-2820, or visit us online at www.AAMDS.org.

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## What’s Inside?

Skim through this table of contents to find the sections you want to read.

### Table of Contents

- **Using This Booklet** ............................................ 2
- **Overview of PNH** ............................................... 3
- **A Closer Look at PNH** ........................................... 5
  - Facts to know about blood .................................... 6
  - What is the complement system? .............................. 7
  - What causes PNH? ........................................... 7
  - What does not cause PNH? ..................................... 8
  - The big question ............................................. 8
- **What are the Symptoms of PNH?** ............................ 10
  - Hemolysis (hi-MOL-uh-suss) .................................. 11
  - Thrombosis (throm-BOE-suss) ................................. 12
  - Low blood cell count .......................................... 14
  - PNH is related to two other bone marrow failure diseases ............................................. 16
- **How Do I Know I Have PNH?** ................................ 18
  - 3 types of PNH blood cells ................................... 18
  - How did my doctor classify my blood cells into these 3 groups? ........................................... 19
  - What other tests may I get? .................................. 19
- **5 Steps to Taking an Active Role in Your Care** ............ 20
  - Find an expert on blood problems who you can work with ............................................. 20
  - Learn all you can about PNH and possible treatment options ........................................... 20
  - Talk with your doctor and other healthcare professionals ............................................. 20
  - Write down your medical information ........................ 21
  - Work with your doctor to make a treatment plan ............................................. 21
- **Living Successfully with PNH** ................................. 23
  - Take care of your body ......................................... 23
  - Take care of your mind ........................................ 25
- **Ways to Treat PNH** ............................................. 27
  - Treating hemolysis (hi-MOL-uh-suss) and anemia ................. 27
  - Treating blood clots ........................................... 34
  - Treating bone marrow failure .................................. 35
  - Bone marrow transplant ....................................... 36
- **About Clinical Trials** .......................................... 40
- **Special Issues for People with PNH** ......................... 42
  - Airplane travel and high altitudes .................................... 42
  - Pregnancy ................................................................ 42
  - Surgery .................................................................. 43
- **More Ways to Get Help** .......................................... 44
- **13 Good Questions to Ask Your Doctor** (tear out sheet) ............................................. 45
Finding out you have PNH can be a lot to take in all at once. PNH is short for paroxysmal nocturnal hemoglobinuria (par-uk-SIZ-muhl nok-TURN-uhl hee-muh-gloe-buh-NYOOR-ee-uh). Reading this booklet will help you and your family get the answers you need. Some people read the book from cover to cover. Others read only the sections they need right now, and then pull the booklet out again at a later date when they want to learn more.

There are many technical medical terms when talking about PNH. We tell you what they mean and how to pronounce them.

For more information about PNH and related diseases, please call us at (800) 747-2820, or visit us online at www.AAMDS.org.

“This booklet does a great job of giving the basics of PNH. I got copies for all my family members and friends because they want to support me the best they can.”

—Carla
Overview of PNH

PNH is a rare and serious blood disease that causes red blood cells to break apart. It can cause different symptoms in different people.

Who gets PNH?

PNH:

• Can occur at any age, but is more common in young adults.
• Cannot be passed down through the genes from parent to child.
• Cannot be passed through germs from person to person.
• Is closely linked to diseases of the bone marrow.

What causes PNH?

A change in a specific gene, called the PIG-A gene, causes PNH. Scientists do not know why this change occurs in some people, but not in others.

What are the symptoms of PNH?

PNH can cause a number of symptoms. These symptoms can vary greatly from person to person.

You may:
• Feel very tired.
• Be in pain. Some people have spasms in their esophagus (i-SOF-uh-gus) – the organ that connects the mouth to the stomach. It is also called the food tube. Others may feel pain in the stomach or belly area.

You may have:
• A low red blood cell count. This is called anemia (uh-NEE-mee-uh).
• Blood clots in a vein. This is called thrombosis (throm-BOE-suss).
• Dark urine.

You may have trouble:
• Getting or keeping an erection (be impotent).
• Swallowing.
How do I find out if I have PNH?

If you have some of these symptoms or have certain bone marrow diseases, your doctor may ask to screen you for PNH. He or she will take a small sample of your blood and send the sample to a lab for tests:

- **CBC** (complete blood count) gives information about the quantity and quality of each type of cell in your blood.
- **Flow cytometry** (sy-TOM-uh-tree) gives information about the proteins on the surface of your blood cells.

5 Steps to Taking an Active Role in Your Care

If you’re like most people with PNH, you want to stay in control as much as possible. Here are some ways to take an active role in your care. Take these 5 steps:

1. Find an expert on PNH who you can work with.
2. Learn all you can about PNH and possible treatment options.
3. Talk with your doctor and other healthcare professionals.
4. Write down your medical information in a notebook or on a computer.
5. Work with your doctor to make a treatment plan.

Ways to Treat PNH

There are a number of treatments for PNH. Here are some of them:

- Blood transfusions (see page 28)
- Growth factors (see page 30)
- Blood thinners (see page 34)
- Eculizumab (Soliris®) (see page 32)
- Bone marrow transplant (see page 36)
“I just found out I have PNH, and I have a ton of questions. I want to know why I got the disease. I want to know what’s going to happen to me. I want to know why my doctor thinks I have PNH. Most of all, I want to know what I can do to stay as well as I can.”

—Tyler

PNH is a rare and serious blood disease that causes red blood cells to break apart. Doctors call this breaking apart hemolysis (hi-MOL-uh-suss). It happens because your blood cells are missing proteins that protect them from your body’s immune system.

When your red blood cells break apart, the hemoglobin (HEE-muh-glo-bun) inside them is released. Hemoglobin is the red part of red blood cells. Its job is to carry oxygen around your body. The release of hemoglobin causes most PNH symptoms.

Where does the name come from?

Here’s where the name “paroxysmal nocturnal hemoglobinuria” comes from:

• **Paroxysmal** means sudden and irregular.
• **Nocturnal** means at night.
• **Hemoglobinuria** means hemoglobin in urine. Hemoglobin is the red part of red blood cells. It makes your urine look dark.

So “paroxysmal nocturnal hemoglobinuria” means sudden, irregular episodes of passing dark colored urine, especially at night or in the early morning. It is important to note that many people with PNH do not have episodes of dark urine, but have other symptoms of PNH.
Facts to Know About Blood

In order to understand PNH, it’s important to know certain facts about blood.

What is blood made of?

Blood is made of blood cells floating in plasma (PLAZ-muh). The plasma is mostly made of water with chemicals in it. These chemicals include proteins, hormones, minerals, and vitamins.

What are the 3 basic types of blood cells?

1. **Red blood cells** make up almost half of blood. They are filled with hemoglobin (HEE-muh-glo-bun). Hemoglobin is the red part of red blood cells. Its job is to carry oxygen around your body.

2. **White blood cells** fight disease and infection by attacking and killing germs that get into your body. There are several kinds of white blood cells, each of which fights a different kind of germ.

3. **Platelets** are small pieces of cell that help blood clot and stop bleeding.

**Healthy bone marrow contains stem cells.**

Bone marrow stem cells mature into red blood cells, white blood cells and platelets.
How are blood cells made?

All 3 types of blood cell are made by stem cells in the bone marrow. Bone marrow is a spongy tissue located inside some bones.

Bone marrow stem cells make copies of (clone) themselves all the time. These cloned stem cells eventually become mature blood cells. When blood cells are fully formed and functional, they leave the bone marrow and enter the blood.

What is the complement system?

The complement system is a group of proteins in the blood. They help support (complement) the work of white blood cells by fighting infections.

These proteins are always active at a very low level. But when bacteria, viruses, and other foreign or abnormal cells get into your body, these proteins become more active (are activated). They work together to attack and destroy the abnormal cells in your body.

Normal red blood cells have a shield of proteins. This shield protects the cells from being attacked by the complement system. The gene in charge of making this protective shield is called PIG-A.

What causes PNH?

PNH occurs because of a change (mutation) in the PIG-A gene of a single stem cell in the bone marrow. Here are the steps that lead to PNH:

1. The abnormal stem cell makes copies of (clones) itself. This leads to a whole population of stem cells that have mutant PIG-A.
2. The abnormal stem cells turn into mature red blood cells that have mutant PIG-A. These are called PNH blood cells.
3. The PNH red blood cells lack the shield of proteins that protect normal red blood cells from the complement system. So they may be attacked and destroyed by the complement system proteins.

Many normal, healthy people have a small number of stem cells with mutant PIG-A. But in people with PNH, these stem cells grow fast and make lots of mature PNH red blood cells.

Some doctors believe this happens because people with PNH have bone marrow that is weaker than normal. Your bone marrow may be weakened because you have aplastic anemia or another bone marrow failure disease (see page 16 for information on aplastic anemia). Weakened bone marrow may also result from a mild bone marrow disease that was never diagnosed.
In general, the more PNH cells you have, the worse your symptoms will be. If 10 out of every 100 red blood cells in your body is a PNH cell, you may have some symptoms of PNH.

What does not cause PNH?

“I have 3 kids and 5 grandkids. I’m worried. Could they inherit PNH from me? How about my wife? Can I give it to her when I kiss her?”

—Roberto

PNH cannot be passed down through the genes from parent to child. It cannot be passed through germs from person to person.

The Big Question

Q: I have PNH. What will happen to me over time? And how long can I expect to live?

A: The course of PNH varies a lot from person to person. You may have only mild symptoms. Or you may have severe symptoms and need medicines or blood transfusions (trans-FYOO-zhunz).

People with PNH live an average of 15 to 20 years after they find out they have the disease. This is just an average, though. Actual survival times vary more widely.

People who develop blood clots in key parts of the body, develop MDS (myelodysplastic syndromes), or AML (acute myeloid leukemia) may have a shorter lifespan. Other people with PNH can live for decades.

The Good News!

New treatments are becoming available. They are helping people with PNH live longer. To learn about ways to treat PNH, see page 27.
PNH is a rare and serious blood disease that causes red blood cells to break apart.

Blood is made of red cells, white cells, and platelets floating in plasma.

Stem cells in the bone marrow can grow into all 3 types of blood cells.

The complement system is a group of proteins in the blood that work together to attack and destroy abnormal cells in your body.

Normal red blood cells have a shield of proteins that protects the cells from being attacked by the complement system. The gene in charge of making this protective shield is called PIG-A.

PNH occurs because of a change in the PIG-A gene of a single stem cell in the bone marrow.
“My skin looks washed out. I feel tired all the time. I get dizzy and out of breath easily. Dr. Ludwig says it’s because I’m anemic.”

—Gina

PNH can cause a variety of symptoms. You may have many of these symptoms, or just one or two. And you may get a new symptom at any point in the course of your illness. Some people have more severe symptoms than others do.

All the symptoms you have with PNH are caused by one of the following:

• **Hemolysis** (hi-MOL-uh-suss) – The breaking apart or destruction of red blood cells.
• **Thrombosis** (throm-BOE-suss) – Blood clots.
• **Low blood cell count** – Not having enough of a type of blood cell.

These health problems can cause some of the symptoms described on pages 10 to 17.

**Hemolysis (hi-MOL-uh-suss)**

Hemolysis is the breaking apart of red blood cells.

**What causes hemolysis?**

Hemolysis happens when the complement system becomes more active and attacks your PNH red blood cells. The complement system usually attacks only foreign objects, such as viruses and bacteria. But because PNH cells are damaged, it sees them as foreign and attacks them too, causing them to burst.
What are the symptoms of hemolysis?

When your PNH red blood cells break apart, their hemoglobin (HEE-muh-glo-bun) is released into your plasma. Hemoglobin is the red part of red blood cells. Its job is to carry oxygen around your body. The release of hemoglobin can cause a number of symptoms:

• Your urine may get darker. Or it may not darken at all.
• You may have a low red blood cell count (anemia). This can make you:
  • Feel very tired.
  • Get headaches.
  • Have trouble breathing when you exercise.
  • Have an irregular heartbeat.

For more information on the symptoms of anemia, see page 14.

What are the symptoms of severe hemolysis?

If you have severe hemolysis, hemoglobin may be quickly released into your bloodstream. This can cause a number of symptoms:

• You may feel very tired.
• The hemoglobin may bind with and remove nitric oxide (NIGH-trik OK-side) from your system.

Nitric oxide helps your muscles stay smooth and relaxed. A shortage of it can cause muscle spasms in certain parts of your body:

• You may have anything from mild to severe pain in your abdomen or belly area.
• You may have spasms in your esophagus (i-SOF-uh-gus). That’s a tube in your throat that goes from your mouth to your stomach. The spasms can make it hard to swallow.
• Males may have trouble getting or keeping an erection (be impotent).

To learn about ways to treat hemolysis and anemia, see page 27.
Thrombosis (throm-BOE-suss)

Thrombosis is a blood clot in a vein. It is often simply called a blood clot.

Who gets blood clots?

At least 1 out of 3 people with PNH get blood clots.

Why are blood clots so common for people with PNH?

Scientists are not sure exactly why people with PNH are more likely to get blood clots. But some believe that PNH patients have abnormal platelets that are too “sticky.” This means the platelets make clots too easily.

Plus, many people with PNH have a shortage of nitric oxide (NIGH-trik OK-side). Nitric oxide helps prevent blood clots by making it harder for platelets to stick together. Hemolysis (hi-MOL-uh-suss) – another symptom of PNH – can cause a shortage of nitric oxide.

To learn more about hemolysis, see page 10.

What are the symptoms of blood clots?

The symptoms of blood clots depend on where the clots occur. People who are otherwise healthy and don’t have PNH sometimes get blood clots in the veins of the leg. But people with PNH tend to get blood clots in other parts of the body, such as in the brain or abdomen (belly area).

A Blood Clot in the Abdomen (Belly Area)

You may get a blood clot in your abdomen, or belly area. That’s the area below your chest and above your hips. Here are some places in the abdomen where you may get a blood clot:

- You may get a blood clot in your spleen.
- You may get a blood clot in the major vein that leaves your liver. This is called Budd-Chiari syndrome (bud-kee-AR-ee SIN-drome).
- Your intestine (bowel) may not get enough blood. This is called ischemia (iss-KEE-mee-uh).
If you have a blood clot in your abdomen:

- You may have fluid and swelling in the belly area. This is called ascites (uh-SITE-eez).
- The area where the clot is may feel warm to the touch.
- The area where the clot is may be painful.

If the blood clot in your abdomen is not treated:

- Part of your intestine may die (dead bowel).
- Your liver may be damaged and stop working.

**A Blood Clot in the Brain**

You may get a blood clot in the veins covering your brain. If this happens:

- You may get a very bad headache.
- Your brain may not work as well as usual. You may have trouble speaking, seeing, or moving parts of your body.

**A Blood Clot in the Skin**

You may get a blood clot in the veins of your skin. If this happens:

- Your skin in that area may get red, puffy, warm, or painful.

**A Blood Clot in the Arm or Leg**

You may get a blood clot in the veins of your arm or leg. If this happens:

- That limb may get warm, puffy, or painful.

**A Blood Clot in the Lung**

Sometimes a blood clot breaks off and travels to your lung. This is called a pulmonary embolism (PULL-muh-nerr-ee EM-buh-liz-um). If you have a pulmonary embolism:

- You may have a sharp pain in your chest. It may get worse when you breathe deeply.
- You may have trouble breathing (shortness of breath). Or you may start breathing fast.
- You may suddenly feel anxious.
- You may cough up some blood.
- You may feel dizzy. You may even faint.
- You may sweat a lot.
If you think you have any symptoms of a blood clot, tell your doctor right away. You may need treatment to save your life.

**How do I find out if I have a blood clot?**

To diagnose a blood clot, your doctor may take pictures of your insides using:

- CT scan (Cat Scan)
- MRI (Magnetic Resonance Imaging)
- Doppler scan
- V-Q Scan (Ventilation-Perfusion Scan)

Or your doctor may order a lab test called D-dimer. It’s also called Fragment D-Dimer, or Fibrin degradation fragment.

To learn about ways to treat blood clots, see page 34.

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**Low Blood Cell Count**

Many people with PNH have a low blood cell count. That means you don’t have enough of one or more types of blood cell.

**Low Red Blood Cell Count**

The most common shortage in people with PNH involves red blood cells.

A low red blood cell count is called anemia (uh-NEE-mee-uh). Red blood cells carry oxygen from your lungs to the rest of your body.

A small number of people with PNH have low counts for other blood cell types, usually because of other bone marrow problems.
Low White Blood Cell Count
A low white blood cell count is called neutropenia (noo-truh-PEE-nee-uh). White blood cells fight infections in the body by attacking and killing bacteria and viruses.

Low Platelet Count
A low platelet count is called thrombocytopenia (throm-buh-sie-tuh-PEE-nee-uh). Platelets help blood to clot and stop bleeding.

What are the symptoms of low blood cell count?
Read the section below to see the symptoms you might expect based on the type of blood cells involved. Check off any signs you are having, and share this list with your doctor.

If you have a very low red blood cell count, you may:

- Feel just a little tired, or very tired.
- Have trouble breathing.
- Feel less alert or have trouble concentrating.
- Have paler than normal skin.
- Have rapid heartbeat and chest pain. This most often happens only in severe cases.
- Have a loss of appetite or lose weight.

If you have a very low white blood cell count, you may:

- Stay sick longer and tend to get fevers when sick.
- Have trouble fighting infections.
- Get skin infections.
- Get sinus infections and a stuffy nose.
- Get lung infections that make breathing hard.
- Get bladder infections that may make it painful to pass urine, or make you urinate more often.
- Get mouth sores.
- Feel tired or have a low energy level.
If you have a **very low platelet count**, you may:

- Bruise or bleed more easily – even from minor scrapes and bumps.
- Get nose bleeds.
- Have bleeding gums, especially after dental work or from brushing your teeth. Be sure to check with your doctor before getting any dental work.
- Get tiny, flat red spots under your skin, which are caused by bleeding. These spots are called petechiae (puh-TEE-kee-ie). These occur more often in the lower legs.

If you have any of the symptoms above, you should let your doctor know.

**PNH is related to two other bone marrow failure diseases**

PNH is related to 2 diseases that cause the bone marrow to stop making cells properly. These bone marrow failure diseases are aplastic (ay-PLASS-tik) anemia and MDS (myelodysplastic syndromes, pronounced my-eh-lo-diss-PLASS-tik SIN-dromez).

**PNH is related to aplastic anemia:**

- More than 10 out of every 100 people with aplastic anemia will develop PNH.
- Some people with PNH will develop aplastic anemia.

**PNH is related to MDS:**

- Between 1 out of 100 and 3 out of 100 people with PNH develop MDS.

**What are the symptoms of aplastic anemia and MDS?**

The symptoms of aplastic anemia and MDS vary, depending on which blood cell counts are low. They are the same as those on pages 15 and 16, including:

- Bleeding
- Fever
- Symptoms of anemia (feeling tired, paler than normal skin)
- Chills
- Signs of infection

If you think you have any of these symptoms, tell your doctor right away.
How do I find out if I have aplastic anemia or MDS?

Diagnosing these diseases is very tricky. Your doctor may:

- Examine a sample of your liquid bone marrow
- Examine a sample of your solid bone marrow
- Order blood tests or other tests

To learn more about aplastic anemia and MDS, please call the Aplastic Anemia and MDS International Foundation (AA&MDSIF) at (800) 747-2820, or visit us online at www.AAMDS.org. We can answer your questions and provide you with educational booklets on both MDS and aplastic anemia.

✔ Review

All the symptoms you have with PNH are caused by one of the following:

- Hemolysis (hi-MOL-uh-suss) – The breaking apart or destruction of red blood cells.
- Thrombosis (throm-BOE-suss) – Blood clots.
- Low blood cell count – Not having enough of a type of blood cell.
“It took a long time to find out what’s wrong with me. To be honest, it’s kind of a relief to know the truth. Now I can put my energy into getting better.”

—Albert

PNH is a rare disease. Many doctors have never seen a case of it. So people with PNH may have symptoms for several years before they get a correct diagnosis.

3 Types of PNH Blood Cells

With PNH, your doctor can usually divide your blood cells into 3 types:

1. PNH I cells, or Type I cells
   These cells respond in a healthy way to the complement system. They are normal cells.

2. PNH II cells, or Type II cells
   These cells are partially sensitive to the complement system. They are missing some of the proteins that protect them from attack.

3. PNH III cells, or Type III cells
   These cells are extremely sensitive to the complement system. Of the 3 groups of cells, these ones break apart most easily. They are missing all the proteins that protect normal cells from attack.

Most people with PNH have mostly Type I and Type III cells. But the amount of each type of cell can vary greatly.
How did my doctor classify my blood cells into these 3 groups?

Your doctor probably used a flow cytometry (sy-TOM-uh-tree) test. This test let your doctor see if any proteins were missing from the surface of your blood cells. It can also be done on certain white blood cells called granulocytes (GRAN-yuh-loe-sites). FLAER is a new type of flow cytometry test.

You may have heard of a test called “Ham’s test” that was used in the past to check for PNH. Because this test does not always give correct results, it is no longer used.

What other tests may I get?

Your doctor may ask you to get other lab tests as well. These include:

- CBC (complete blood count).
- Ferritin (FER-ut-un) test to check your iron levels.
- Tests of your solid bone marrow (through a bone marrow biopsy).

Your doctor may also look for high levels of:

- A pigment called bilirubin (bil-i-ROO-bun).
- An enzyme called LDH (lactate dehydrogenase, pronounced LAK-tose dee-high-DROJ-uh-nase).
- Young red blood cells in your bone marrow.

✔️ Review

Tests for PNH include the following:

- **Flow cytometry** (sy-TOM-uh-tree) gives information about the proteins on the surface of your blood cells.
- CBC (complete blood count) gives information about the quantity and quality of each type of cell in your blood.
"My doctor, my family – they are here to help and support me. But I’m the one in charge. I’m the one who has to make the tough choices.”

—Sara

If you’re like most people with PNH, you want to stay in control as much as possible. Here are some ways to take an active role in your care. Take these 5 steps:

1. Find an expert on PNH who you can work with.

Ask your regular doctor or your insurance company for the name of a hematologist (hee-muh-TOL-uh-jist) who treats people with PNH. Or call a teaching hospital near you that is linked with a medical school to find this specialist.

Choose a doctor who is treating people with PNH or similar bone marrow problems. He or she is more likely to be aware of new medicines and treatments. Make sure your doctor clearly answers your questions, explains all of your treatment options, and includes you in making decisions.

2. Learn all you can about PNH and possible treatment options.

We congratulate you for reading this booklet and sharing it with your friends and family. To get other free materials on PNH, call us at (800) 747-2820, or visit us online at www.AAMDS.org.

3. Talk with your doctor and other healthcare professionals.

Ask as many questions as you wish. If you are not sure what the doctor meant, ask again.

Remember, your doctor is there to help you!
Here are 13 good questions to ask. There is also a tear out sheet with these questions on page 45 that you can take with you to your doctor.

**About my treatment**

1. What have other people with a similar disease and treatment gone through?
2. What are all my treatment options?
3. What treatment option do you recommend for me? Why?
4. How likely am I to get better with the treatment?
5. Has this treatment been used a lot (standard)?
   Or is it a new or experimental treatment?
6. How long will the treatment take to work? When will I know if it is working?
7. Can my disease return, even after successful treatment?

**About my medicine**

8. Has the medicine been FDA approved for treating PNH?
9. How do I take the medicine? How often do I need to take it?
10. How long will I need to take the medicine?
11. What are the common side effects of this medicine?
    What can be done to control them?
12. What are the long-term side effects of this medicine?
13. How much does it cost? Is it covered by my insurance?

**4. Write down your medical information.**

Keep a notebook with questions to take to your doctor visits. Ask a family member to come along to help you take notes. Or track your health information on a computer. You can also write down your symptoms and treatment details in the back of this booklet.

**5. Work with your doctor to make a treatment plan.**

Work closely with your doctor to find the best treatment plan for your specific type of PNH. Together, look at all the risks and benefits. This way, you can make an informed choice about your treatment.
✔ Review

To take an active role in your health care:
■ Find an expert on PNH who you can work with.
■ Learn all you can about PNH and possible treatment options.
■ Talk with your doctor and other healthcare professionals.
■ Write down your medical information in a notebook or on a computer.
■ Work with your doctor to make a treatment plan.
“I used to love downhill skiing. But that kind of workout is too much for me these days. I’ve had to find ways to move that work for the body I’m in now.”

—Camilla

Your lifestyle plays a key role in managing your PNH. By taking good care of your body and mind, you allow yourself to be as healthy as possible.

Take Care of Your Body

Eat a healthy diet.

There is no specific diet that makes PNH better or worse. Experts recommend a well-balanced diet with plenty of fruits and vegetables. Your doctor can help you find the best eating plan for you.

Your doctor will probably make sure you get enough iron and a B-vitamin called folate (FOE-late) in your diet. You may need to take man-made forms of these. Check with your doctor before taking any medicines, supplements, vitamins, or herbs.

Get the right amount of exercise.

It’s good for your body to get some form of regular exercise. But since you have PNH, you may not have much energy to stay active. Plus, you may need to take special precautions. Ask your doctor what amount of exercise and what kinds of activity would work for you!

If you have a very low red blood cell count, you should avoid:

- Any activity that hurts your chest, makes it very hard to breathe, or makes your heart beat fast.
- Going up too high in the mountains. High altitude can make this health problem worse.
If you have a **very low platelet count**, you should avoid:

- Any activities that could hurt you. Ask your doctor about safe ways to stay active. Tell your doctor if you get a bad headache or pain that could mean you are bleeding, or any of the other symptoms mentioned on page 16.

If you have a **very low white blood cell count**, you should protect yourself from germs (infection):

- Carry a lotion that kills bacteria when you go out. Use it if you can’t find soap and water.
- Floss and brush your teeth often to prevent the need for dental work. Dental work can cause infection.
- Keep minor infections from getting serious. Tell your doctor if you have a fever or feel very tired. These can be early signs of infection.
- Make sure your food is not too hot. Burns can cause infection in your mouth.
- Stay away from crowds and sick people.
- Wash your hands often.

**Special diet for very low white count**

If your white count is very low, your doctor may ask you to avoid certain foods that can make you sick. This is called a neutropenic (noo-truh-PEE-nik) diet. Take these steps to keep germs out of your food:

- Avoid eating raw meats and fish, such as sushi.
- Do not eat any cheeses that say “aged” on the label.
- Drink only beverages that have been pasteurized to kill germs. Milk you buy from the supermarket is fine to drink. Stay away from any fermented drinks such as homemade wine, cider, root beer, ale, vinegar, and unpasteurized milk.
- Stay away from buffets, salad bars, and crowded restaurants. Germs could end up on your food.
- Wash and peel fresh fruit and vegetables very well before eating them.
Take Care of Your Mind

Different people react in different ways to having PNH. Read what these PNH patients have to say about their experience.

“*I’m an upbeat person*, and so I was surprised that I felt so down after I learned I had PNH. I joined a support group. These weekly meetings help me manage better. Now if I feel low I do something fun – take a walk with my neighbor or work in my garden. It works!”

—*Lori*

“I was glad to finally learn there was a name for my health problem. It is easier for me to cope now that I know what is happening. I read as much as I can to stay up on the latest research.”

—*Alex*

“*Funny as this sounds* — I feel I have become a stronger person since I found out I have PNH. I’m keeping the faith and finding I am actually closer to my husband and children. I enjoy life and stay active — I won’t let PNH take over my life.”

—*Nina*
PNH can be hard to cope with, but you don’t have to do it alone. The AA&MDSIF is here to help. Contact us to:

- Get connected to other people who have PNH through the Peer Support Network.
- Get support from our Patient Information Specialists.
- Order our booklets on managing treatment decisions and other subjects related to living with PNH.

To contact us, call (800) 747-2820. Or visit us online at www.AAMDS.org.

Here are some other ways to take care of your mind:

- Join a support group. Examples of online support groups for PNH include:
  - www.marrowforums.org
  - www.pnhdisease.org
- Learn more about PNH.
- Speak with a Chronic Illness Counselor.
- Take an anti-depressant medicine, if your doctor says it will help you.

✓ Review

To live successfully with PNH:
- Take care of your body.
- Take care of your mind.
There are lots of treatments for PNH, and they all have their pros and cons. Dr. Jacob and I have been going through them and trying to find the best choices for me.”

—Rhonda

PNH is considered chronic (KRON-ik). That means it lasts for a long time. The only known cure is a bone marrow transplant. Other treatments are designed to ease symptoms and prevent problems. A small number of people say their PNH went away on its own over time.

Before getting any treatment…

Talk with your doctor about the risks and benefits. That way you can make an informed choice about which treatment is best for you.

Treating Hemolysis (hi-MOL-uh-suss) and Anemia

For most people with PNH, the most common problem is anemia caused by hemolysis. Here are some treatments for hemolysis and anemia.
Blood Transfusions

In a blood transfusion, whole blood or parts of blood from a donor are put right into your bloodstream. This can improve your anemia. Your doctor will look at your symptoms to decide if you need a transfusion.

The 2 types of transfusion available for PNH patients are:
• Red blood cell transfusion.
• Platelet transfusion.

Red Blood Cell Transfusion

A red blood cell transfusion is the more common type of transfusion used to treat people with PNH. It usually takes 2 to 5 hours to complete. Here’s what to expect.

Before the transfusion:
• If your immune system is not healthy (immunocompromised, pronounced i-myoo-no-KOM-pruh-mized) or you are a candidate for a bone marrow transplant, the donor blood cells may be treated with radiation first. This irradiation (ir-rade-ee-AY-shun) prevents graft-versus-host disease (GVHD) (see page 38).
• Your doctor may ask you to take Tylenol® and Benadryl® before the transfusion to help prevent fever, chills, and allergic reaction.

During the transfusion:
• Your blood will be tested to make sure it matches the donor blood. This usually takes about 1 hour.
• Your doctor will check your vital signs – temperature, heartbeats per minute, breaths per minute, and blood pressure.
• Your doctor will give you an IV in your vein. You will get 1 to 3 units of red blood cells without plasma (packed red blood cells).

After the transfusion:
• If you took Tylenol® and Benadryl®, you may feel drowsy.
Side Effects of Red Blood Cell Transfusion

A red blood cell transfusion carries the risk of certain side effects:

• If you get many transfusions (more than 50), iron may build up in your body (iron overload). This can put your organs at risk. Iron overload can be treated with iron chelators (KEE-lay-tors). For more information on iron overload and iron chelation see page 33.

• You may have an allergic reaction.

• Your red blood cells may break apart (hemolysis).

• Very rarely, you may be infected with a virus.

Platelet Transfusion

If you don’t have enough healthy platelets in your blood or if you are bleeding, you may get a platelet transfusion. Platelets live only about 8 to 10 days. So a platelet transfusion lasts for just 3 to 4 days.

Side Effects of Platelet Transfusion

Platelets are more likely than red blood cells to cause an immune response. So you are more likely to have side effects like chills and fever.

Your body is quicker to develop antibodies against platelets than against red blood cells. This can cause platelet transfusions to fail.

Iron Therapy

Hemolysis (hi-MOL-uh-suss) can lead to a shortage of iron in your body. This can make it hard for your bone marrow to make red blood cells. So unless you are getting regular red blood cell transfusions, you probably need to take iron pills. Ask your doctor how much iron you need. Iron therapy is especially important for young women who are not getting blood transfusions.

Side Effects of Iron Therapy

At first, taking iron pills can cause hemolysis, which leads to dark urine. This happens because your bone marrow is making more red blood cells. Some of them break apart because you have PNH.

Iron pills can also cause an upset stomach. If you have a severe stomach problem, you can get your iron by IV. There is a very small risk of allergic reaction when iron is given this way.
Folate and Folic Acid

Folate is a B-vitamin that is found in fresh or lightly cooked green vegetables. It helps your bone marrow make normal blood cells. When your bone marrow has to make more cells, it needs a larger supply of folic acid.

Most people get enough folate in their diet. But since you have PNH, it’s a good idea to take 1 mg each day of a man-made form of folate called folic acid.

Growth Factors

Growth factors are chemicals in your body. They cause your bone marrow to make blood cells.

Man-made forms of some growth factors are available. They can reduce the need for red blood cell transfusions.

Red Blood Cell Growth Factors

Erythropoietin (i-rith-row-POY-uh-tun), or EPO for short, is a growth factor made by your kidneys. It causes your bone marrow to make more red blood cells.

Man-made forms of erythropoietin are available. Here are some of them:

- Epogen® and Procrit® are taken once a week as a shot. This is called a subcutaneous (sub-kyoo-TAY-nee-us) injection.
- Darbapoietin (Aranesp®) is taken every 2 to 3 weeks.

Some people with PNH have naturally high levels of erythropoietin. So these drugs don’t help them. In addition, some doctors believe that EPO increases the risk of hemolysis (hi-MOL-uh-suss) and clotting in PNH patients because it causes the bone marrow to produce more PNH red blood cells.
White Blood Cell Growth Factors

If you don’t have enough healthy white cells in your blood, your doctor may ask you to take a white blood cell growth factor to improve your immune system. White blood cell growth factors cause your bone marrow to make white blood cells. These are the most common ones:

- G-CSF (granulocyte colony-stimulating factor) is sold under the names Filgrastim® and Neupogen®.
- GM-CSF (granulocyte macrophage colony-stimulating factor) is sold under the names Leukine® and Sargramostim®.

Androgens

Androgens are natural male hormones that can cause your bone marrow to make more red blood cells, which improves anemia. They are more likely to be used if you have other bone marrow failure problems besides PNH. This is an older treatment for bone marrow failure that is rarely used because of the side effects. Some androgens include:

- Danazol (Danacrine®)
- Fluoxymestrone (Halotestin®)
- Oxymetholone
- Stanazolol (Winstrol®)
- Testosterone

Side Effects of Androgens

The main side effect of androgens is that they tend to make you more masculine.

If you are a man:

- Your breasts may grow.
- Your prostate may grow.

If you are a woman:

- Hair may grow on your face.
- You may get more muscular.
- Your clitoris may grow.
- Your voice may deepen.
Other Side Effects of Androgens

Androgens can cause other side effects as well. They can:

- Cause acne.
- Increase liver enzymes. This can cause yellow skin (jaundice) and liver damage.

Eculizumab (Soliris®)

Eculizumab is the only drug approved by the FDA (U.S. Food and Drug Administration) and the EMEA (European Medicines Evaluation Agency) to treat PNH. It works by making your complement system less active.

Benefits of Eculizumab

Eculizumab has many benefits. It has been found to:

- Improve anemia (see page 14).
- Reduce or get rid of the need for transfusions (see page 28).
- Reduce the breaking apart of red blood cells (hemolysis) over both the short and the long term (see page 10).
- Reduce the risk of blood clots (see page 12).

Eculizumab does not help increase white blood cell count or platelet count.

How You Take It

Your doctor will give you an IV at the office or at a special center. The procedure usually takes about 35 minutes.

You will probably get an IV once a week for the first 4 weeks. Starting in the 5th week, you will get a slightly higher dose of eculizumab every 2 weeks.

Common Side Effects of Eculizumab

If you take eculizumab, you may:

- Feel nauseous or sick to your stomach.
- Get a runny nose or colds.
- Have a headache or back pain.
Rare Side Effects of Eculizumab

If you take eculizumab, you have a higher risk of being infected with the bacteria that can cause a type of meningitis.

Some doctors worry that if PNH red blood cells are no longer destroyed, they may build up in the blood. In theory, if you were to stop taking eculizumab, large numbers of those cells could be destroyed. However, several patients stopped taking Eeculizumab gradually, and they did not have any severe problems as a result.

Prednisone

Prednisone is a steroid that can decrease hemolysis (hi-MOL-uh-suss) caused by PNH. It may make the complement system less active. It may also increase counts of white blood cells and platelets in some people.

Prednisone does not stop all hemolysis, however. And it does have a lot of side effects, especially if taken for a long time. So doctors disagree about whether it should be used by people with PNH.

Most people who take prednisone for a long time use it only every other day. This schedule eases the most severe side effects of prednisone.

If your hemolysis is worse than normal, your doctor may recommend that you take 60 mg for 3 to 4 days, then gradually cut back to the regular schedule.

Iron Chelation (kee-LAY-shun) to Treat Iron Overload

You should get your blood iron level checked regularly if you get red blood cell transfusions often. That’s because the transfusions can cause you to have too much iron in your blood. This can lead to a condition called iron overload. Iron overload can hurt your heart and other organs. Iron overload can start to become a problem after as few as 20 blood transfusions.

If your blood iron level, or ferritin (FER-i-tin), is between 1,000 and 2,000, this is considered high and treatment may be necessary.
If you have iron overload, your doctor may ask you to take an iron chelator (KEE-lay-tor) to get the extra iron out of your body. The FDA (U.S. Food and Drug Administration) has approved 2 iron chelators to treat iron overload in the U.S.

- Deferasirox (Exjade®) is a tablet that you mix with juice or water and swallow. It is taken once a day. Because it can be taken by mouth, it is also called an oral iron chelator.
- Deferoxamine (Desferal®) is a liquid that you can take by injection, either under your skin or into your muscle. Or you can pump it slowly into your vein. It is usually taken 3 to 7 times a week.

An oral iron chelator called deferiprone (Ferriprox®) is approved for use in Europe and elsewhere, but not the U.S. It is usually taken 3 times a day (morning, afternoon, and night). The number of tablets taken per day depends on your weight and how much iron you have in your body.

**Treating Blood Clots**

Blood clots are very dangerous. If you have a blood clot, you need to get help right away. Here are some ways blood clots are treated.

**Blood Thinners**

Blood thinners are the most common way to treat blood clots. They decrease the ability of blood to clot.

Blood thinners are also called anticoagulation (ant-i-koe-ag-yuh-LAY-shun) drugs, or anticoagulants (ant-i-ko-AG-yuh-lunts).

Here are some of them:

- Enoxaparin (Lovenox®) is taken as a shot 1 or 2 times per day.
- Heparin (Calciparine® or Liquaemin®) is taken as a shot or by IV. Your doctor will keep an eye on the dose to prevent bleeding problems.
- Warfarin (Coumadin®) is swallowed. The foods you eat can affect the level of warfarin in your blood. Your doctor will keep an eye on the dose to prevent bleeding problems and to make sure the Warfarin in your blood is at a high enough level.

Some doctors think some people with PNH who are more at risk of blood clots should take blood thinners to prevent clots. Others disagree.
tPAs (Tissue Plasminogen Activators)

tPAs can break up an existing blood clot for people with PNH - especially when used as soon as possible after the clot occurs. Ask your doctor about how they can work best for you.

Eculizumab (Soliris®)

Studies have shown that eculizumab can reduce the risk of blood clots too. To learn more about eculizumab, see page 32.

Other Drugs

Some drugs, such as aspirin and ibuprofen, stop platelets from working well. This can help stop blood clots. But doctors don’t know how well they prevent or treat clots in people with PNH. **Take these drugs only if your doctor says you should.**

Treating Bone Marrow Failure

Bone marrow failure is a condition in which your bone marrow does not make enough healthy cells. In people who have PNH, bone marrow failure is most likely to be caused by aplastic anemia. So the treatment for bone marrow failure is the same as that for aplastic anemia.

Immunosuppressive (i-myoo-no-suh-PREH-siv) Therapy

If you have bone marrow failure caused by aplastic anemia, your doctor will probably first try to treat it by lowering your immune response. This is called immunosuppressive therapy. You may take an immunosuppressive drug. The standard treatment for aplastic anemia is:

• Anti-thymocyte globulin (ATG or ALG for short) is given by IV.
• Cyclosporine is a pill.

Other immunosuppressive drugs are being tested for treatment of aplastic anemia. But we don’t know how well they work for people with PNH. For more information about immunosuppressive therapy, please visit us online at www.AAMDS.org.

In addition to immunosuppressive drugs, people with aplastic
anemia also receive supportive care to help increase blood cell counts. These treatments include:

- Blood transfusions (see page 28).
- Growth factors (see page 30).

**Bone Marrow Transplant**

If the treatments described on pages 28 to 35 have not worked to stop hemolysis (hi-MOL-uh-suss), clotting, or bone marrow failure, then a bone marrow transplant may be the next step.

In this procedure, healthy bone marrow stem cells and other bone marrow cells are taken from a donor. These cells are given to you by IV. The donor’s marrow makes its way through your blood and into your bones. The donor marrow is called a graft.

Bone marrow transplantation is the only way to cure PNH. But it carries many risks, including death.

**Finding a matched related donor**

**Q:** My sister has PNH and needs a bone marrow transplant. How do I find out if I can be her donor?

**A:** Your sister’s transplant will be more likely to succeed if she can get a matched related donor. This involves finding a close relative – usually a brother or sister – with matched HLA (human leukocyte-associated) antigens. Leukocytes (LOO-kuh-sites) are white blood cells that protect your body from infection and remove harmful substances. HLA antigens are proteins on the surface of these cells. A blood test (or a cheek swab) can be used to show whether your HLA antigens match hers.

**Finding a matched unrelated donor**

If you cannot find a matched related donor, your doctor can help you search for a matched unrelated donor, or MUD for short. About 70 out of 100 people cannot find a matched related donor.

**If you may be a candidate for a bone marrow transplant in the future, start looking for a donor right away. It can take time to find an unrelated donor if no related donor is available.**
If there’s no perfect match …

Even if you cannot find a perfectly matched donor, you may still be able to receive bone marrow from a partially matched donor. It depends on the type of mismatch.

Getting a Bone Marrow Transplant

*Kelly’s Story*

“After searching for almost 2 years, I finally found a bone marrow donor. Here’s the story of my transplant.

The first step was making room for the new bone marrow. Dr. Thompson destroyed my old marrow with a mixture of chemotherapy and radiation. This took 2 days, although it can take up to a week.

Finally, the big day came. Dr. Thompson gave me an IV. The donor marrow went into my vein.

Soon the cells from the marrow entered my bones and started making new, healthy blood cells. Dr. Thompson called this process engraftment and said it usually takes 2 to 4 weeks. During this time, she kept a close eye on my blood counts. And she gave me medicines to protect me from infections while my new immune system was growing.

It’s been a year since my transplant. I’ve had to limit my activities and lifestyle somewhat because of the risk of infection. But Dr. Thompson says I can relax and enjoy my life now. And that is just what I am doing!”
What is the success rate of bone marrow transplants?

- The success rate for all bone marrow transplants with matched related donors is about 40 out of 100.
- The success rate for all bone marrow transplants with matched unrelated donors is about 20 out of 100.

Researchers believe these success rates may be much higher for PNH patients. And new research on bone marrow transplants is continuing to improve success rates.

Bone marrow transplants work best if you:

- Are younger than 55 years old.
- Have a matched related donor.
- Have no other health problems.

The chance of success goes down if:

- There was a big lag between diagnosis and transplant.
- You have had many blood transfusions (trans-FYOO-zhunz).
- You have had serious infections that weakened your body.

What can go wrong?

Putting marrow from one body into another is a complicated process. Here are some things that can go wrong.

Your body rejects the graft.

Some bone marrow transplants fail because your body rejects the donor graft. This is more likely if the graft came from an unrelated donor.

The graft rejects and attacks your organs.

Other transplants fail because your donor’s marrow reacts to or rejects tissues in your body. This condition is called GVHD (graft-versus-host disease).

GVHD’s symptoms can range from mild to deadly. In some cases, it can be prevented or treated with medicine that lowers your immune response (immunosuppressive therapy).

GVHD is more likely if:

- You are an older adult.
- Your donor was not a close relative.
- Your donor was not a perfect match.
Other Types of Transplants

A standard bone marrow transplant is not the only way to replace your marrow. Here are some other types of transplants.

PBSCT (Peripheral Blood Stem Cell Transplant)

In this procedure, healthy stem cells are collected from the donor’s bloodstream and given by IV. Some researchers think PBSCT is less likely to cause graft rejection, but more likely to cause GVHD.

Mini (Non-myeloablative) Transplants

Mini transplants use a less harmful form of chemotherapy than the form used in standard bone marrow transplants, which reduces the risk of side effects caused by chemo and the risk of infection during the transplant. This makes mini transplants a better choice for older adults. The procedure may carry a higher risk of GVHD, though.

Scientists are trying to find out if mini transplants can help younger people who have not been helped by standard treatments. Mini transplants are still considered experimental.

Cord Blood Transplants

In this procedure, doctors inject stem cells from umbilical cord blood into your body. The cord blood is collected right after the birth of a baby and kept very cold until needed.

Even if the cord blood is not a perfect match, it carries a lower risk of severe GVHD than mismatched bone marrow or peripheral blood stem cells. But the number of stem cells available in cord blood is small. So normally only people who weigh less than 40 kg (80 pounds) qualify for cord blood transplants. Scientists are looking into the use of cord blood for people who weigh more.

✔ Review

The main types of treatment for PNH include:

- Blood transfusions
- Blood thinners
- Eculizumab (Soliris®)
- Growth factors
- Bone marrow transplant
Scientists are always looking for new and better ways to treat PNH. They are conducting controlled, clinical trials, also called research studies. The studies are often done at university medical research centers around the world.

These studies:

- Compare new treatments or new combinations of treatments with standard ones.
- Help scientists learn more about standard treatments.
- Test the safety and effectiveness of new treatments.

You may want to explore whether you may be eligible for a clinical trial if:

- Standard treatments have not made your PNH better.
- Your PNH came back after standard treatments.
- You are not satisfied with how well standard treatments work.

**Before entering a clinical trial, discuss the possible risks and benefits with your doctor.**

For more information on clinical trials looking for PNH patients, visit the AA&MDSIF Web site at www.AAMDS.org. Or call (800) 747-2820 and ask to speak with a Patient Education Specialist.
✔ Review

Clinical trials:

- Compare new treatments or new combinations of treatments with standard ones.
- Help scientists learn more about standard treatments.
- Test the safety and effectiveness of new treatments.
Special Issues for People with PNH

“I take airplanes for my job. My doctor told me I should switch to the train. She says my blood cell counts are too low to fly.”

—Peter

Because you have PNH, everyday events can be more risky for you than for healthy people. Here are some examples.

Airplane Travel and High Altitudes

The farther you get from Earth, the less oxygen there is. If you have anemia, flying in an airplane or going up high may cause a shortage of oxygen. It may also cause chest pain.

Before you do either of these things, it’s a good idea to:

- Get a red blood cell count.
- Get treatment for your anemia (blood transfusions or growth factors).

If you do fly, remember to:

- Drink plenty of water.
- Get up and walk around every hour or 2 if it is safe to do so.

Pregnancy

Pregnancy is possible with PNH, but it’s not a good idea. It carries risks for both mother and child.

A woman with PNH faces a number of risks during pregnancy:

- Her blood may have fewer healthy cells.
- Her bone marrow may make fewer healthy cells.
- She is more likely to get blood clots. Most doctors place pregnant women with PNH on blood thinners, to prevent clots. But warfarin (Coumadin®) cannot be used during the first trimester, since it may affect the way the fetus develops.
• She is more likely to get preeclampsia (pree-i-KLAM-see-uh). That’s a dangerous condition that causes very high blood pressure and can put both mother and baby at risk.
• She may need red blood cell transfusions (trans-FYOO-zhunz) more often.

A baby whose mother has PNH has a greater risk of:
• Being born too soon.
• Dying in the womb.
• Having a low birth weight.
• Having delayed growth and development.

Still, about 1 out of 3 babies whose mothers have PNH do not have any of these problems.

If you do get pregnant, look for a PNH specialist and an OB (obstetrician) who specializes in high-risk births.

**Surgery**

Surgery can also be risky for people with PNH. Surgery:
• Makes the complement system more active, which can cause hemolysis (hi-MOL-uh-suss).
• Increases the risk of getting blood clots.
• Can cause serious bleeding in people with a low platelet count. Platelet transfusions may be needed before surgery.

If you do get surgery, it’s a good idea to:
• Make sure your PNH specialist talks with your surgeon.
• Take the blood thinner Heparin (Calciparine® or Liquaemin®) as soon as possible after surgery, if you don’t have a low platelet count and if your doctor says you should.

**✔ Review**

Since you have PNH, these everyday events may be more risky for you:
- Airplane travel and high altitudes
- Pregnancy
- Surgery
The Aplastic Anemia & MDS International Foundation (AA&MDSIF) is here to help. We provide the following services:

- Free support through our Patient Education Specialists.
- Free educational materials on many topics related to PNH.
- Conferences for patients and family members.
- Events and training for healthcare professionals.
- Clinical trials information.
- Newsletters and E-bulletins with important information and updates.

Contact us today. Here’s how:

- Call us: (800) 747-2820
- Email us: help@AAMDS.org
- Go to our website: www.AAMDS.org

Remember – you are not alone. We are standing by to support you in any way we can.
13 Good questions to ask your doctor (tear out sheet)

About my treatment

1. What have other people with a similar disease and treatment gone through?

2. What are all my treatment options?

3. What treatment option do you recommend for me? Why?

4. How likely am I to get better with the treatment?

5. Has this treatment been used a lot (standard)? Or is it a new or experimental treatment?

6. How long will the treatment take to work? When will I know if it is working?

7. Can my disease return, even after successful treatment?
About my medicine

8. Has the medicine been FDA approved for treating PNH?

9. How do I take the medicine? How often do I need to take it?

10. How long will I need to take the medicine?

11. What are the common side effects of this medicine? What can be done to control them?

12. What are the long-term side effects of this medicine?

13. How much does it cost? Is it covered by my insurance?
Important information I want to remember:

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