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# FOCUS ON

FALL 2024

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# THE LATEST ON

## PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

### REST ASSURED, TREATMENT WORKS

Are you on treatment for PNH? A large new study underscores just how effective complement inhibitors are. Researchers analyzed the results of 27 studies that included 912 people with PNH—some on treatment and others not. They found that within about 6 months, people on these medications had significantly lower levels of LDH, an enzyme in the blood that shows your blood vessels are breaking apart and your disease is active. The drugs also slashed the need for blood transfusion and greatly improved people's reports of tiredness.

SOURCE: *Therapeutic Advances in Hematology*

## 30 to 40

Most common ages to get a PNH diagnosis.

SOURCE: National Library of Medicine

## 1 in 4

Number of people with PNH who have dark urine during the night or in the morning.

SOURCE: Orphanet

## 1 in 3

Number of White Americans and Asian Americans with PNH who get a blood clot.

SOURCE: National Library of Medicine

### PNH AND BLOOD CLOTS: WHO'S AT RISK?

Blood clots are the most common and most serious complication of PNH. But it can be hard for doctors to know who's at greatest risk. The results of a new study may help. Researchers compared two groups of people with PNH: those with a history of blood clots and those without. They discovered a couple of different lab values that may serve as red flags alerting doctors of the risk for future clots. Past clots are a factor, too. This improved understanding of risk factors for blood clots may help your doctor better assess your unique risk and provide more customized care. Ask your doctor where you stand.

SOURCE: *Annals of Hematology*



# BEYOND PNH

Patient portrayal.

Ask your doctor about FABHALTA, a groundbreaking, FDA-approved oral PNH treatment for adults.

**Improved hemoglobin (Hb) levels with the first FDA-approved oral treatment for adults with PNH.** In a study of 62 people receiving FABHALTA and 35 people receiving C5 inhibitors (SOLIRIS® or ULTOMIRIS®), 82% of people taking FABHALTA had an increase in Hb  $\geq 2$  g/dL vs 0% of people taking C5 inhibitors. 68% of people taking FABHALTA had Hb levels  $\geq 12$  g/dL vs 0% of people taking C5 inhibitors. These results were achieved in the absence of red blood cell transfusions after 24 weeks.

Approved Use

What is FABHALTA?

FABHALTA is a prescription medicine used to treat adults with paroxysmal nocturnal hemoglobinuria (PNH). It is not known if FABHALTA is safe and effective in children.

Important Safety Information

What is the most important information I should know about FABHALTA?

FABHALTA is a medicine that affects part of your immune system and may lower your ability to fight infections.

- FABHALTA increases your chance of getting serious infections caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type b. These serious infections may quickly become life-threatening or fatal if not recognized and treated early.
  - You must complete or update your vaccinations against *Streptococcus pneumoniae* and *Neisseria meningitidis* at least 2 weeks before your first dose of FABHALTA.
  - If you have not completed your vaccinations and FABHALTA therapy must be started right away, you should receive the required vaccinations as soon as possible.
  - If you have not been vaccinated and FABHALTA must be started right away, you should also receive antibiotics to take for as long as your health care provider tells you.
  - If you have been vaccinated against these bacteria in the past, you might need additional vaccinations before starting FABHALTA. Your health care provider will decide if you need additional vaccinations.
- Vaccines do not prevent all infections caused by encapsulated bacteria. **Call your health care provider or get emergency medical care right away if you have any of these signs and symptoms of a serious infection:**
  - Fever with or without shivers or chills
  - Fever with chest pain and cough
  - Fever with high heart rate
  - Headache and fever
  - Confusion
  - Clammy skin
  - Fever and a rash
  - Fever with breathlessness or fast breathing
  - Headache with nausea or vomiting
  - Headache with stiff neck or stiff back
  - Body aches with flu-like symptoms
  - Eyes sensitive to light

**Your health care provider will give you a Patient Safety Card about the risk of serious infections.** Carry it with you at all times during treatment and for 2 weeks after your last dose of FABHALTA. Your risk of serious infections may continue for a few weeks after your last dose of FABHALTA. It is important to show this card to any health care provider who treats you. This will help them diagnose and treat you quickly.

**FABHALTA is only available through a program called the FABHALTA Risk Evaluation and Mitigation Strategy (REMS). Before you can take FABHALTA, your health care provider must:**

- Enroll in the FABHALTA REMS program.
- Counsel you about the risk of serious infections caused by certain bacteria.
- Give you information about the symptoms of serious infections.
- Make sure that you are vaccinated against serious infections caused by encapsulated bacteria and that you receive antibiotics if you need to start FABHALTA right away and you are not up to date on your vaccinations.
- Give you a **Patient Safety Card** about your risk of serious infections.



## DISCOVER A DIFFERENT PNH TREATMENT

Learn more about this disease and how FABHALTA works at [www.FABHALTA.com](http://www.FABHALTA.com)

On the site, you'll find:



Important disease education



Key data about FABHALTA including Safety and Side Effects



Financial Support



Novartis Patient Support™

No matter where you are on your treatment journey, we're here to help.

Who should NOT take FABHALTA?

Do not take FABHALTA if you:

- Are allergic to FABHALTA or any of the ingredients in FABHALTA.
- Have a serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, or *Haemophilus influenzae* type b when you are starting FABHALTA.

Before you take FABHALTA, tell your health care provider about all your medical conditions, including if you:

- Have an infection or fever.
- Have liver problems.
- Are pregnant or plan to become pregnant. It is not known if FABHALTA will harm your unborn baby.
- Are breastfeeding or plan to breastfeed. It is not known if FABHALTA passes into your breast milk. You should not breastfeed during treatment and for 5 days after your final dose of FABHALTA.

**Tell your health care provider about all the medicines you take,** including prescription and over-the-counter medicines, vitamins, and herbal supplements. Taking FABHALTA with certain other medicines may affect the way FABHALTA works and may cause side effects. Know the medicines you take and the vaccines you receive. Keep a list of them to show your health care provider and pharmacist when you get a new medicine.

**If you have PNH and you stop taking FABHALTA,** your health care provider will need to monitor you closely for at least 2 weeks after stopping FABHALTA. **Stopping treatment with FABHALTA may cause a breakdown of red blood cells due to PNH.**

**Symptoms or problems that can happen due to breakdown of red blood cells include:**

- Decreased hemoglobin level in your blood
- Blood in your urine
- Shortness of breath
- Trouble swallowing
- Tiredness
- Pain in the stomach (abdomen)
- Blood clots, stroke, and heart attack
- Erectile dysfunction (ED)

**It is important that you take FABHALTA exactly as your health care provider tells you to lower the possibility of breakdown of red blood cells due to PNH.**

What are the possible side effects of FABHALTA?

FABHALTA may cause serious side effects, including:

- See "What is the most important information I should know about FABHALTA?"
- Increased cholesterol and triglyceride (lipid) levels in your blood.** Your health care provider will do blood tests to check your cholesterol and triglycerides during treatment with FABHALTA. Your health care provider may start you on a medicine to lower your cholesterol if needed.

**The most common side effects of FABHALTA in adults include:**

- Headache
- Nasal congestion, runny nose, cough, sneezing, and sore throat (nasopharyngitis)
- Diarrhea
- Pain in the stomach (abdomen)
- Infections (bacterial and viral)
- Nausea
- Rash

Tell your health care provider about any side effect that bothers you or that does not go away. These are not all the possible side effects of FABHALTA. Call your doctor for medical advice about side effects.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088.

**Please see Important Facts About FABHALTA on the following page.**

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## IMPORTANT FACTS ABOUT FABHALTA® (iptacopan)

### USE

FABHALTA is a prescription medicine used to treat adults with paroxysmal nocturnal hemoglobinuria. It is not known if FABHALTA is safe and effective in children.

- FABHALTA is one capsule taken twice a day, every day, with or without food.

### WARNINGS

**FABHALTA is a medicine that affects part of your immune system. FABHALTA may lower the ability of your immune system to fight infections.**

- FABHALTA increases your chance of getting serious infections caused by encapsulated bacteria**, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type b. These serious infections may quickly become life-threatening or fatal if not recognized and treated early.
  - You must complete or update your vaccinations against *Streptococcus pneumoniae* and *Neisseria meningitidis* at least 2 weeks before your first dose of FABHALTA. If you have not completed your vaccinations and FABHALTA must be started right away, you should receive the required vaccinations as soon as possible.
  - If you have not been vaccinated and FABHALTA must be started right away, you should also receive antibiotics to take for as long as your health care provider tells you.
  - If you have been vaccinated against these bacteria in the past, you might need additional vaccinations before starting FABHALTA. Your health care provider will decide if you need additional vaccinations.
  - Vaccines do not prevent all infections caused by encapsulated bacteria. **Call your health care provider or get emergency medical care right away if you have any of these signs and symptoms of a serious infection:** Fever with or without shivers or chills; fever with chest pain and cough; fever with high heart rate; headache and fever; confusion; clammy skin; fever and a rash; fever with breathlessness or fast breathing; headache with nausea or vomiting; headache with stiff neck or stiff back; body aches with flu-like symptoms; eyes sensitive to light.
- FABHALTA is only available through a program called the FABHALTA Risk Evaluation and Mitigation Strategy (REMS). Before you can take FABHALTA, your health care provider must:**
  - Enroll in the FABHALTA REMS program
  - Counsel you about the risk of serious infections caused by certain bacteria
  - Give you information about the symptoms of serious infections
  - Make sure that you are vaccinated against serious infections caused by encapsulated bacteria and that you receive antibiotics if you need to start FABHALTA right away and you are not up to date on your vaccinations
  - Give you a **Patient Safety Card** about your risk of serious infections

### OTHER IMPORTANT INFORMATION

- Do not take FABHALTA if you:** Are allergic to iptacopan or any ingredients in FABHALTA; have a serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, or *Haemophilus influenzae* type b when you are starting treatment.
- Increased cholesterol and triglyceride (lipid) levels in your blood.** Your health care provider will do blood tests to check your cholesterol and triglycerides during treatment with FABHALTA. Your health care provider may start you on a medicine to lower your cholesterol if needed.

**Before you take FABHALTA, tell your health care provider about all your medical conditions, including if you:**

- Have an infection or fever
- Have liver problems
- Are pregnant or plan to become pregnant. It is not known if FABHALTA will harm your unborn baby
- Are breastfeeding or plan to breastfeed. It is not known if FABHALTA passes into your breast milk. You should not breastfeed during treatment and for 5 days after your last dose of FABHALTA

### COMMON SIDE EFFECTS

**The most common side effects** that occurred in patients treated with FABHALTA were headache, nasal congestion, runny nose, cough, sneezing, and sore throat (nasopharyngitis), diarrhea, pain in the stomach (abdomen), infections (bacterial and viral), nausea, and rash. **These are not all the possible side effects. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.**

**This information is not comprehensive.**

How to get more information: Talk to your health care provider or visit [www.fabhalta.com](http://www.fabhalta.com) to obtain the FDA-approved product labeling.



SCAN ME

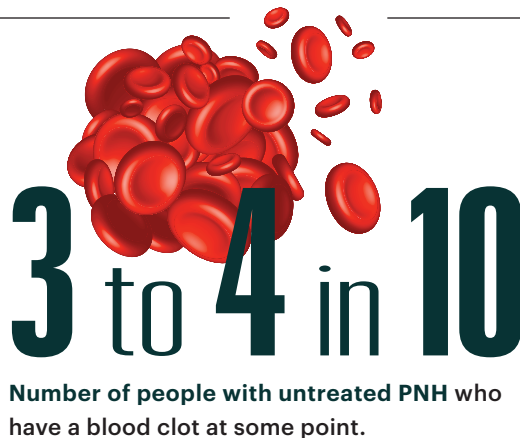
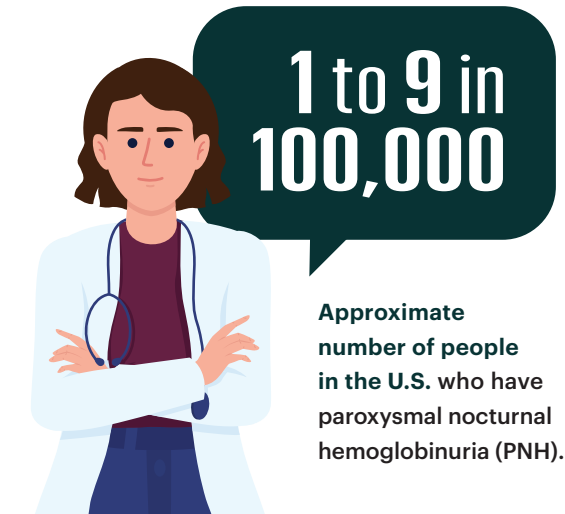
For more on how your race affects the risk of complications with this blood disorder, read PNH in Black and Hispanic Populations.

Use your smartphone camera to activate the QR code.

# STATS & FACTS

By Sonya Collins

Reviewed by Neha Pathak, MD, WebMD Lead Medical Editor



SOURCES: Orphanet, National Library of Medicine, Clinical and Applied Thrombosis/Hemostasis



# HOW PAROXYSMAL NOCTURNAL HEMOGLOBINURIA AFFECTS YOUR BLOOD

## BRUSH UP ON THE BASICS

By Kendall K. Morgan

Reviewed by Brunilda Nazario, MD, WebMD Chief Physician Editor, Medical Affairs

You probably know that paroxysmal nocturnal hemoglobinuria (PNH) is a blood disorder. While PNH comes with fatigue and other, more troubling symptoms, many people notice that their urine is stained red or brown, says Robert A. Brodsky, MD, a hematologist at Johns Hopkins Medicine in Baltimore. This is where the condition gets its name.

The reason this happens is that your immune system is attacking and damaging the red blood cells that carry oxygen through your bloodstream. Your discolored urine is a sign of the destruction taking place in your blood. You're most likely to see this in the morning when you wake up. But the process of red cells breaking down is happening all the time.

You'll also have fewer healthy red

blood cells than you should, a condition called anemia, as those cells break down prematurely. Even when the breakdown of your red blood cells doesn't happen too much, loss of those cells may cause fatigue, rapid heartbeat, headaches, chest pain, and trouble breathing when you exercise. As it gets worse, you may have trouble swallowing, spasms in your belly and esophagus, and other signs. PNH can leave you prone to getting blood clots, too.

"Before there was effective therapy, blood clots in the abdomen and brain were the leading cause of death," Brodsky says. Fortunately, doctors now treat PNH with medicines that prevent those potentially life-threatening complications.

### WHERE IT STARTS

Each year, about 6 in every 1 million people—most often men and women between the ages of 30 and 40—learn they have PNH. It affects men and women equally and tends to happen in people with other conditions that affect bone marrow, where blood cells are made. The trouble arises when a genetic change pops up in the stem cells that make your blood cells. This change happens in a gene called PIGA, which you need to make anchors that attach other proteins to your blood cells.

"This mutation leads to the absence of several proteins on the [blood] cell

surface," Brodsky explains. "Two of these proteins are important for controlling part of our immune system known as complement. The absence of these proteins means that the body's own immune system will destroy red blood cells."

The complement system normally cleans up damaged cells. Its job is to help you heal after you get hurt or have an infection. But when you have PNH, your complement system sees the defects in your blood cells and destroys them.


### WHAT ABOUT OTHER BLOOD CELLS?


Your body has other types of blood cells, too, including white blood cells—part of your immune system—and platelets. So what happens to them? Brodsky explains that your white blood cells will miss the same surface proteins as red blood cells. They also get attacked by the complement system. The difference is that white blood cells can repair the damage in ways that red blood cells can't.

Platelets, which allow your blood to clot after you get a cut, don't fare as well. While you'll still have platelets, they'll be defective in ways that may encourage too much clotting. Thankfully, treatments that block the complement system can now stop the damage to red blood cells and platelets before it happens. As a result, you can now expect to live just as long with PNH as you would without it.

## BLOOD CELLS 101


Your blood is mostly fluid, known as plasma, with three main types of blood cells floating in it.

 **Red blood cells have hemoglobin to carry oxygen through your body.**


 **White blood cells fight infections.**

 **Platelets clot your blood to stop you from bleeding.**

## DEFINING PNH

 **Paroxysmal: sudden or happening in irregular episodes**

 **Nocturnal: at night**

 **Hemoglobinuria: hemoglobin (the red part of your blood) in urine**

# WHAT TRIGGERS PNH SYMPTOMS

**EVEN WHEN YOU TAKE MEDICATION, YOU MIGHT OCCASIONALLY SEE A RETURN OF YOUR SYMPTOMS. LEARN HOW YOU CAN MANAGE IT.**

By Sonya Collins

Reviewed by Neha Pathak, MD, WebMD Lead Medical Editor

Many people with paroxysmal nocturnal hemoglobinuria (PNH) need treatment with medications called complement inhibitors to keep the condition under control. These come in the form of twice-monthly IV infusions or twice-daily pills. For most people, the medications greatly improve symptoms.

“But even on complement inhibitors, you can get breakthrough events or symptoms caused by a variety of different mechanisms,” says Gloria F. Gerber, MD, a hematologist and assistant professor of medicine at Johns Hopkins Medicine in Baltimore.

Here’s what you need to know about what might trigger breakthrough symptoms and how to avoid these situations.

## RECOGNIZE A PNH FLARE

If an event triggers a PNH flare, you’ll most likely see some of the same symptoms you had when you first got diagnosed. You might have signs of anemia, like feeling tired or weak. You could also have dark urine like you did before.

## IT’S TIME FOR YOUR MEDICINE

If you run late on a dose of your medication—whether you forget to take your daily pills or you miss an infusion appointment—you could see your symptoms come back.

You may not even have to miss a dose to see symptoms. “In some patients, the drug levels get low at the end of the 2 weeks before your next infusion,” Gerber says. “But now that we have other drugs, switching to one of those can certainly help.”

## YOU’RE HAVING SURGERY

Surgery is a major stressor on the body and can cause a flare of PNH symptoms. If you need surgery, there’s no way to avoid that. But you might need to have your medication adjusted beforehand or—if you get



IV medications—schedule your surgery shortly after a dose.

“Talk to your hematologist before scheduling the surgery, or have your hematologist involved in some way,” Gerber says.

## YOU’VE GOT AN INFECTION

Living with PNH, you might need to be extra cautious about infections. The flu, COVID-19, or any other type of infection can trigger symptoms.

“General infection precautions could be helpful—good handwashing and staying away from people who you know could be sick.”

## YOU’RE PREGNANT

If you’re not already on treatment for PNH before you get pregnant, you will need to be during pregnancy. As for those already on treatment, some need more medication during this time. That might mean a higher dose or a shorter interval between doses.

“You may need increased vigilance and collaboration between the members of your care team,” Gerber says, “so make sure you’re being followed closely by your hematologist and by obstetrics and maternal-fetal medicine.”



# BE PROACTIVE ABOUT YOUR CARE

## STAY ON TOP OF YOUR PNH MANAGEMENT

By Rachel Reiff Ellis

Reviewed by Neha Pathak, MD, WebMD Lead Medical Editor

You are your best advocate when it comes to living with a rare disease like paroxysmal nocturnal hemoglobinuria (PNH). Consider yourself not only an active participant in your health care, but a project manager. Here's how.

### PREPARE FOR APPOINTMENTS

Gather your thoughts before your visit with your doctor. This will help you make good use of the face-to-face time you have and help you not to forget anything that's been concerning you.

"Write down all your questions for your provider before you go," says Bhumika J. Patel, MD, a hematologist-oncologist with Prisma Health in Greenville, SC. "Make sure you find out and record

your blood counts while you're there, so you know how you're doing on treatment and can ask about alternatives if needed."

### TRACK YOUR SYMPTOMS





Keeping tabs on your symptoms helps both you and your doctor see the big picture of your treatment journey. If you're wondering how and where to start, look for ready-made resources like symptom tracker apps for your smartphone or premade tools online.

"The Aplastic Anemia and MDS International Foundation (AAMDS) has a PNH Toolkit," Patel says. "Not only does it help you learn more about your disease, but you can also use it to chart your progress on



## QUESTIONS FOR YOUR DOCTOR

Come prepared with prompts to learn more about your PNH.

-  **How will I know treatment is working?**
-  **How do I get in touch with you between visits?**
-  **How do I access my health information?**
-  **Are there clinical trials I should know about?**

KLEBERCORDEIRO/WIA GETTY IMAGES



treatment and record questions that come up along the way.”

Tracking symptoms not only helps you see how well treatment is working, but it can also give you a record of the side effects you may be having from treatment, too.

### COMMUNICATE WELL AND OFTEN

Honesty and transparency are crucial as you work with your doctor to treat your PNH. If you don’t understand something your doctor is saying, ask for clarification. If you still don’t, ask again.

“We want you to leave feeling like you know where things stand and what the next steps are,” says LeRoy Keiser, MD, hematologist-oncologist at Providence Medical Group in Santa Rosa, CA. “We’re not offended if you ask us to repeat information.”

It’s a good idea to bring a trusted friend or family member along with you to visits, too, so you have another set of ears on hand.

Every doctor’s office is different when it comes to doctor-patient contact after hours. Many now have patient portals where you can send and receive messages from your doctor, often within 24 hours. Ask your clinic at the first visit about office communication policies so you know what to expect when you have a concern outside of your visits.

“Your doctor will have a protocol for handling emergencies outside of office hours,” Keiser says. For nonemergency concerns, some doctors prefer you call the nurse line, others prefer a portal message. Some will even give out their personal phone number—it just depends on the doctor.”

## AT A GLANCE

Steps toward proactive PNH care.

- 💧 Set an agenda for your visits.
- 💧 Be open and honest.
- 💧 Ask for clarification when you don’t understand.
- 💧 Keep tabs on your blood counts.
- 💧 Know how to get in touch.





SCAN ME 

Flip through our slideshow on **Tips for Caring for Yourself with PNH**. Use your smartphone camera to activate the QR code.

# ALTERNATIVE AND COMPLEMENTARY THERAPIES FOR PNH

## LEARN HOW MEDITATION, ACUPUNCTURE, AND MORE CAN HELP

By Kendall K. Morgan

Reviewed by Neha Pathak, MD, Lead Medical Editor



You may wonder whether complementary and alternative therapies could help your PNH and its symptoms. At most large medical centers, you may find these offered through clinics or centers dedicated to integrative medicine, says Mikhail Kogan, MD, a specialist in geriatrics and palliative medicine at the GW Center for Integrative Medicine in Washington, DC.

“Integrative medicine combines any therapy that’s evidenced, effective, and safe to combine with standard treatment,” Kogan says. “An important difference in integrative medicine is that we believe the body has a strong, innate capacity to help and heal itself. If we can address what is hindering the healing, the body will know what to do. We’re working with the body to optimize and make it stronger to combat the illness better.”

### EXPLORING YOUR ALTERNATIVES

According to the Aplastic Anemia and MDS International Foundation, complementary and alternative medicine practices have reported benefits as sources for hope, relaxation, improved mood, body awareness, coping ability, and

more. They’re also popular, with almost 4 in 10 people having tried them in some form. While these approaches aren’t included in official guidelines for treating PNH and your hematologist might not bring it up, don’t hesitate to ask what’s available at your health center or elsewhere in the community.

Complementary and alternative approaches can include vitamins, herbs, and other supplements. Check to be sure that it’s OK to try any new supplement along with your treatment regimen. If you’re not sure where to start, Kogan suggests you go to a specialist in integrative or functional medicine for

ANDREA WYNER/VIA GETTY IMAGES



additional testing in search of more information about what may be affecting your health. For example, he says that many people with blood disorders don't have enough vitamin K. A doctor can check to see if you are deficient for vitamin K or another nutrient. He notes that they also may look to see if you show signs of exposure to toxins, including heavy metals, and may benefit from treatments to address those.

### TACKLING PAIN, STRESS, AND MORE

Kogan says there's also plenty of evidence in support of approaches including acupuncture, massage, tai chi,

and yoga. These varied approaches can help with pain, trouble sleeping, anxiety, stress, and more. See if a medical center in your community offers training in mindfulness-based stress reduction (MBSR). Whenever possible, Kogan recommends you see an experienced MBSR practitioner at a practice that's integrated with your hematology team.

Even if alternative approaches can't treat your PNH, Kogan says, "at a very minimum, diagnosis [of a blood disorder] causes anguish and anxiety. MBSR or other stress-reducing techniques can be very helpful."

### PRACTICE GOOD SELF-CARE

Carlos M. De Castro III, MD, a hematologist-oncologist at Duke Health in Durham, NC, recommends good self-care including a focus on nutrition, more fruits and vegetables, and less processed foods and meats. He says regular exercise and relaxation techniques are helpful, too. If you're feeling emotional distress, let your doctor know.

"If someone is feeling a lot of stress or anxiety, they need to get help," De Castro says. "This can either be from a physician or nurse, or they can get help from family, friends, or other support mechanisms, such as a pastor or priest. If these don't help, then referral to a therapist might be needed."

### COMPLEMENTARY AND ALTERNATIVE APPROACH CHECKLIST

Ask your doctor if it's OK to try complementary and alternative approaches along with your PNH treatment, including:

- ✓ Herbal medicines or teas
- ✓ Probiotics
- ✓ Vitamins or other supplements
- ✓ Meditation
- ✓ Yoga
- ✓ Deep-breathing exercises
- ✓ Chiropractic or osteopathic medicine
- ✓ Physical therapy
- ✓ Massage
- ✓ Reiki
- ✓ Ayurvedic or Chinese medicine



# HAVE HOPE

## MY JOURNEY WITH PNH TREATMENT

By Maegan Voss

Reviewed by Neha Pathak, MD, WebMD Lead Medical Editor

The road to my PNH diagnosis was a long one. After 10 years of extreme, constant fatigue and chest pains severe enough to send me to the ER (only to be tested and sent home with no explanation) and a separate diagnosis of hemophagocytic lymphohistiocytosis (HLH), finally a flow cytometry test gave me the validation I had been searching for.

I felt both vindicated and relieved. I had proof of what I already knew: I was disabled. And I was excited that I finally had something to look up, research—and treat.

### A PATH FORWARD

Before my diagnosis, I thought my only option for relief was going to be a bone marrow transplant, which I didn't want to do. But then I learned there were medications that can treat PNH.

My doctor started me on one of the most common medications for PNH at the time, an immunosuppressive drug that stops red blood cells from being destroyed. Unfortunately, that drug didn't work well for me. I had been so hopeful that I'd be able to see results immediately after starting a treatment, but it took some time to get things established.



SCAN ME

For more on treatment, read **PNH: Motivation When Treatment Gets Hard**. Use your smartphone camera to activate the QR code.

### FINDING A FIT

What did work for me—and still does—is a twice-a-week injectable drug that I give myself in the fatty part of my belly. I used to have a pump with a lot of wiring that I had to wear around my neck and have it infused, which was kind of a pain. When I traveled, I had to bring lots of equipment along. This newest injector simply sits on my belly. I hit a button and it injects the medication, which takes about an hour.

Medication options for PNH have made huge strides in the past few years, and more options are available. Things are changing and getting easier. For example, if I were



really needle squeamish and I didn't feel comfortable doing the injection myself, there are other choices that involve a nurse or infusion center. So if you try a medication and it doesn't seem like it's working—for any reason—continue the conversation with your oncologist until you find what helps you.

### NEW LEASE ON LIFE

I can't state enough how much it has dramatically changed my life for the better to find a treatment for my PNH. The injections have abated most of my symptoms. I'm still more tired than the average

person, but my fatigue is much better. My doctor and I are working on additional solutions for that.

With treatment, I've stayed out of the emergency room for over a year. I don't have hemolytic episodes with chest pain. And my counts don't drop nearly as much as they used to. I do still have episodes, but my body is able to overcome those much more easily on the infusions.

I'm still disabled, but my quality of life is vastly better. I'm still very cautious about infection and mask up when I leave the house, but I'm *able* to leave the house. And meet new friends and live my life. It's been a blessing.



## FACING THE FUTURE

### Maegan's Tips

- + **Advocate for yourself.** Keep the conversation going with your doctors—and your body.
- + **Learn your limits.** Take things slowly and respect the journey.
- + **Have hope.** If you've been living in pain or been tired, treatment will help you do so much more.

# LIFE AFTER A BONE MARROW TRANSPLANT

## KNOW WHAT TO EXPECT IN THE WEEKS OR MONTHS AHEAD

By Kendall K. Morgan

Reviewed by Neha Pathak, MD, WebMD Lead Medical Editor

A bone marrow transplant is the only cure for paroxysmal nocturnal hemoglobinuria (PNH). But it's also a grueling procedure with serious risks. Now that other treatment options are available, your doctor isn't likely to recommend it unless your other treatments aren't working or your blood counts drop so low that your life is in danger.

"[Bone marrow transplant] is a big procedure," says Neil Dunavin, MD, a hematologist at the University of California San Francisco Health. "It takes months to recover."

The transplant procedure requires high doses of chemotherapy and sometimes radiation to kill your bone marrow cells and make way for new ones. As a result, you'll be prone to infections and feeling poorly for a while. You'll need to stay in the hospital at first, with nurses checking on you often.

"The blood counts typically recover in 2 to 3 weeks," Dunavin says. "That's when people are generally allowed to leave the hospital and start the outpatient recovery part of things."

### HOME AGAIN

Once home, you'll likely return to your clinic two or three times a week for blood checks. You'll take as many as 10 different medicines to help you along.

"You're building a new immune system that will get stronger," Dunavin says. "You're trying to find the balance. You don't want your immune system to be too strong too fast and cause graft vs. host disease. You don't want it too suppressed to cause complications from infections."



### REACHING THE END GOAL

During this time, you'll need to take precautions to avoid infections and will likely feel fatigued. While a walk outdoors is great, Dunavin says, you'll primarily spend your time at home or in the clinic. Ask your doctor about any dietary or other precautions you'll need to take. It usually takes at least 3 months after transplant before one could consider going back to work part-time, he says.

The process of recovery will be gradual as your blood counts come back up and your new immune system gets stronger. Your doctor will monitor you to figure out when you can stop taking so many medicines. By 1 year, you may be able to stop treatments, which can help your energy and stamina return, Dunavin says. The upside is, at the end of it, you can expect to be cured of your PNH.

"Transplant is usually used in more severe circumstances," Dunavin says. "If a blood condition isn't causing [serious] problems, you might not be recommended for transplant. The goal is to get to the other side and function better than before and not have the condition come back."

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