NOVARTIS

FABHALTA® ▼
iptacopan

Patient and Caregiver Treatment Guide

This guide is designed to help you understand your treatment with FABHALTA® ▼ (iptacopan)

Get the support you need with

replenishEd
pnh education & support

As a patient starting treatment with Fabhalta® (iptacopan) you are invited to enrol in the **replenishEd patient support program**. By joining **replenishEd** you will have the option to receive:

- Educational emails on managing PNH with oral therapy
- lonthly phone calls with a haematology nurse
- SMS reminders about medication

Please scan the QR code to enrol in the replenishEd patient support program, or go to: www.replenished-pnh.com.au

Trouble signing up? Call 1800 290 652 or email support@replenished-pnh.com.au

▼ There is additional monitoring in place for FABHALTA® (iptacopan) to help quickly identify new side effects. If you experience any side effects, talk to your doctor, nurse or pharmacist/other healthcare professional. This includes any possible side effects not listed in this guide. If you have any questions or concerns about FABHALTA®, speak with a healthcare professional.

AU-28070 | October 2025

Getting started with your treatment guide

You have recently been prescribed FABHALTA® for the treatment of **paroxysmal nocturnal haemoglobinuria (PNH)**. This guide will help you to understand your **new treatment** and will share **practical tips and advice** to guide you along your treatment journey.

Don't worry if it seems like a lot to take in – **you are not alone**. Within this treatment guide you will find **sources of support**, including recommended patient groups to connect with other people with PNH. This guide also has a **section for caregivers** so that they can understand the **best ways to support you on your treatment journey**. Finally, it can be your reference guide that you always go back to if you have any questions about your treatment.

What's in my treatment guide?

What happens in PNH?

1. **Normal red blood cells (RBCs) have a protective shield** of proteins to defend against any attack from the complement system (part of your immune system).

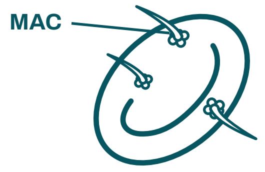
2. In your body, **RBCs are missing key proteins that create this protective shield**. This leaves them vulnerable to attack.

3. The complement system is overactive in PNH and **forms a membrane attack complex (MAC) that punctures holes into unprotected RBCs**, causing them to fall apart.

4. The destruction of RBCs is known as **haemolysis**. It is the reason why you may experience symptoms such as **tiredness, dark or red urine** (from haemoglobin in the blood), **shortness of breath** and **pain**.

1. Normal RBCs
Have a protective shield

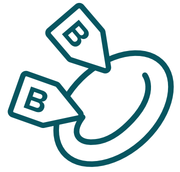
2. RBCs in PNH
Lack key proteins needed to protect them from damage



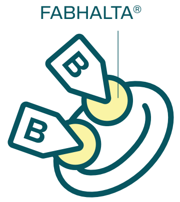
3. Complement attack
The complement system's MAC punctures holes into unprotected RBCs

4. PNH RBC destruction (haemolysis)
PNH RBCs are destroyed and the contents released in the liquid part of the blood known as plasma

How does FABHALTA® work in my body?



1. Factor B is key in PNH



2. FABHALTA® blocks Factor B

3. FABHALTA® reduces haemolysis

1. The complement system (part of your immune system) attacks unprotected RBCs. **Factor B controls complement system activity** by initiating a domino effect which results in a membrane attack complex (MAC) punching holes in RBCs. This causes RBCs to fall apart.
2. FABHALTA® blocks Factor B, which **helps to stop the complement system from attacking the RBCs**.
3. By stopping the complement system attacking RBCs, **FABHALTA® protects your RBCs and reduces the number of RBCs that are destroyed through haemolysis** in your veins and organs.

How to keep the risk of haemolysis low

Because of the constant threat of haemolysis from complement system attack, it's very important to take your treatment as your healthcare team has prescribed to control your symptoms.

As FABHALTA® blocks part of your immune system, it can make some serious infections more likely. This means you will need to **receive vaccinations** against certain bacterial infections **before you start treatment** with FABHALTA®.

Learn more about the vaccinations you need in the **Vaccination Guide**

What's the evidence behind FABHALTA®?

Your new treatment has been tested in several clinical trials to assess if it is effective and safe for people with PNH.

One of these trials was APPLY-PNH. Everyone in the trial had received previous PNH treatments (eculizumab or ravulizumab, anti-C5 for short) but they still had some residual anaemia despite their anti-C5 treatment.

Some people stayed on their previous anti-C5 treatment in the trial, while others switched from their previous treatment to FABHALTA®.

The trial compared the effects of the two types of treatment on haemoglobin levels after 24 weeks. It used two primary (main) endpoints to do this: improvement of haemoglobin levels and normal haemoglobin levels.

After 24 weeks:[1–3]

People with clinically meaningful **improvements* in haemoglobin levels**

FABHALTA® 82.3%

Anti-C5 2.0%

People achieving **normal or near-normal haemoglobin levels****

FABHALTA® 68.8%

Anti-C5 1.8%

The primary endpoints:

The two primary endpoints in APPLY-PNH were the proportion of patients who had a ≥20 g/L increase in haemoglobin from baseline in the absence of red blood cell transfusions and the proportion of patients with haemoglobin ≥120 g/L in the absence of red blood cell transfusions.

* **Clinically meaningful improvement**.
A change that makes a positive or noticeable improvement to a patient. In the case of this clinical trial, it meant patients' haemoglobin levels increased by ≥20 g/L or more from where they were at the start of the clinical trial.

** **Near normal haemoglobin levels**.
Patients achieving haemoglobin levels ≥120 g/L. Normal haemoglobin levels vary, but are generally between 120–160 g/L for women and 130–180 g/L for men.

What's an endpoint?
A clinical endpoint is the outcome that shows the direct benefit of a drug.

References: 1. Peffault de Latour R, et al. Blood. 2022;140(S2):LBA-2. **2.** Risitano AM, et al. HemaSphere. 2023;7(S3):189–190. **3.** Risitano AM, et al. EHA 2023, 8–11 June; Frankfurt, Germany. Oral S182.

Getting to know your treatment

How should I take FABHALTA®?

If not advised otherwise by your treating healthcare team, you should **take FABHALTA® by mouth, two times a day**

It can be **taken with or without food**

You may find it easier to remember to **take your doses when you wake up in the morning** and **before you go to bed**

Why do I need to stick to my dosing schedule?

FABHALTA® was designed to be taken twice a day so that it can work continuously to prevent haemolysis. Missing doses will reduce your protection against haemolysis. That's why you need to adhere to the dosing schedule.

If you are struggling to stick to your dosing schedule, here are a few tips to help you remember:

- ✓ Use your **adherence prompt stickers** around the home as a daily reminder. These can be found in your patient starter kit
- ✓ **Create a schedule** in your calendar
- ✓ **Set a reminder** on your phone. The **patient support programme** offers a text **message (SMS) reminder service** to help you **stay on track** with your treatment, refills and vaccine boosters
- ✓ **Discuss your treatment with your caregiver, friends and family** so they can remind you to take your medication
- ✓ replenishEd pnh education & support

  Enrol in the **replenishEd patient support program to receive SMS reminders**

  replenished-pnh.com.au
- ✓ Try to take your medication at the **same time each day** or combine it with another daily routine such as brushing your teeth
- ✓ **Keep your medication in an obvious place** such as by your bedside, that way you'll be reminded to take it as soon as you wake up and when you go to bed each night

Advance planning for repeat prescriptions

If you are planning any change from your routine, such as a holiday or time away from home, it's important to make sure you don't run out of your prescribed treatment during that time. You might wish to consider carrying spare doses with you to cover one or two extra days. You can speak to your healthcare team/pharmacist in advance if you need to change the number of capsules for your next prescription.

How long will I need to take my medication?

PNH is a lifelong condition and it is expected that you will need to take your new treatment for a long time. Your healthcare team will regularly monitor your condition to check that the treatment is having the desired effect.

If you have questions about how long you will need to take FABHALTA®, talk to your healthcare team.

What should I do if I miss a dose?

If you miss a dose, or doses, it's important that you take one dose of FABHALTA® as soon as possible, even if it's shortly before your next dose is due. You can then carry on as normal with your regular dosing schedule. PNH is a disease that requires continual treatment. To help manage the symptoms of PNH, it's important to make sure that you always take your treatment as advised by your healthcare team.

What should I do if I take too much of my medication?

If you have accidentally taken too many capsules or if someone else accidentally takes your medicine, talk to your healthcare team immediately.

Will I have any side effects from my FABHALTA® treatment?

As with all medicines, side effects can occur. Although not everyone experiences them, there is a chance that you may experience some unwanted side effects while taking your treatment.

In clinical studies, the most common side effects reported by more than 1 in 10 people taking FABHALTA® were:

- Common cold (upper respiratory tract infections)
- Headache
- Diarrhoea

Some people taking FABHALTA® may also experience a decrease in their blood platelet count. This is a common side effect that can happen in more than 1 in 100 people taking FABHALTA®. It's not usually something to worry about as in most cases it's mild and short-lived.

Some other common side effects include:

- Urinary tract infection
- Persistent cough or irritation of the airways (bronchitis)
- Fewer platelets in the blood (thrombocytopenia) which may cause bleeding or bruising more easily than normal
- Dizziness
- Pain in the stomach (abdomen)
- Nausea (feeling sick)
- Joint pain (arthralgia)

Uncommon side effects include:

- Lung infection (with chest pain, cough and fever)
- Hives (urticaria)

What are platelets?

Platelets are small cell fragments found in your blood. They help your blood form clots, which is important to control bleeding. A low platelet count means your body doesn't have enough platelets to form clots. This may cause bleeding or bruising more easily than normal.

What should I do if I get side effects from my FABHALTA® treatment?

Whilst no one wants to have side effects from their treatment, just remember that it isn't unusual to experience them. What's most important is that you speak to your healthcare team immediately, so that they can advise you on how to proceed.

Should I stop taking my treatment if I experience side effects?

Stopping your treatment with FABHALTA® can make your condition worse and may increase your risk of red blood cell breakdown (haemolysis). It is important that you adhere to the scheduled treatment regimen. Do not stop taking FABHALTA® without talking to your healthcare team first.

If your healthcare team decides to stop your treatment, you will be monitored closely for at least 2 weeks for any signs of haemolysis due to PNH. Your healthcare team may prescribe a different PNH medicine or have you restart FABHALTA® treatment.

If you stop treatment, be aware of signs or symptoms of haemolysis, including:

Lower level of haemoglobin in your blood

Tiredness

Dark or red urine

Pain in your stomach (abdomen)

Shortness of breath

Blood clots (thrombosis)

Trouble swallowing

Erectile dysfunction

If you experience any of these symptoms or problems after stopping treatment, please contact your healthcare team immediately.

Navigating your treatment journey

How can I help optimise my treatment?

Your PNH will need continual therapy. You can play a part in managing your disease and therapy by engaging in a few helpful tasks.

These include:

Attend regular check-ups with your healthcare team

This will help your healthcare team to understand how well you are managing with your treatment.

Please refer to the **appointment planner** in your patient starter kit for further details.

Know what signs to look out for in case of side effects with your treatment

Making a note of side effects you experience will help your healthcare team to manage your side effects and decide on your ongoing treatment.

Stay on top of your treatment by using the treatment diary

The treatment diary is a great way to monitor your treatment progress, track your symptoms and record your key measurements from tests and check-ups.

Look out for signs and symptoms of serious infection

Your treatment works by disrupting the body's natural defences against bacterial infection. This means that you may have an increased risk of serious infection for diseases such as meningitis, pneumonia (severe lung infection) and sepsis (blood poisoning).

If you experience any of these signs and symptoms, you must contact your healthcare team immediately:

- **Fever**
  - with or without shivers or chills
  - with chest pain and cough
  - with breathlessness/fast breathing
  - with high heart rate
  - with a rash (this may be harder to see on brown or black skin)
  - with a headache
- **Headache**
  - with a stiff neck or stiff back
  - with nausea or vomiting
- **Confusion**
- **Body aches with flu-like symptoms**
- **Clammy skin**
- **Eyes sensitive to light**

Your patient safety card contains important safety information

When you are first prescribed FABHALTA®, you will be given a **patient safety card**.

This card contains important safety information about the risk of infection while taking FABHALTA® and what to do if you experience certain signs or symptoms.

Keep this card with you at all times during treatment, and for a period of 2 weeks following your last FABHALTA® dose in case of an emergency. You should tell all members of your healthcare team that you are being treated with FABHALTA®.

If you have not received a patient safety card, please contact your healthcare team.

FABHALTA®▼
iptacopan

Patient safety card

Important safety information for patients taking FABHALTA® (iptacopan)

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions.

Planning for different life events or changes in circumstances

It's understandable that you may have a change from your daily routine at times. You may have a long holiday booked, have to travel for work, or perhaps the clinic or pharmacist will be closed for a period of time due to a public holiday. It's important that you take your treatment as prescribed to help keep your PNH under control.

Here are a few tips to help you stay on track if you have a change in circumstance:

Plan repeat prescriptions in advance

If you're going away on holiday, be sure that you give yourself enough time to request and collect your medication so that you can continue to take it twice a day, every day, as prescribed while you're away and when you return.

Keep track of how many capsules are left in your packet

Avoid waiting until you are down to your last few capsules before requesting another prescription. Why not fill out your repeat prescription as soon as you have 10 days' worth of capsules left in your packet.

Be prepared for surgery or pharmacy closures

Always request your repeat prescription well in advance of finishing your packet of capsules when you know the surgery or pharmacy will be closed e.g., for Christmas or bank holidays.

Switching to FABHALTA® from a different therapy

If you are switching from a different PNH treatment to FABHALTA®, there may be a short period of time in which you are at risk of haemolysis.

It's important that you start your new treatment with FABHALTA® at the right time to minimise this risk. Although your healthcare team will be able to advise you of the timings to follow, it can be helpful for you to know the recommended timings for dosing.

If you require any vaccinations, you will need to have these at least 2 weeks before starting treatment with FABHALTA® – learn more about this in the **Vaccination Guide**

Timings for dosings are different depending on the treatment that you were previously prescribed:

Last dose of **eculizumab** or **ravulizumab**

1 week

You should start your FABHALTA® treatment no later than **1 week after the last dose of eculizumab**

6 weeks

You should start your FABHALTA® treatment **no later than 6 weeks after the last dose of ravulizumab**

If you are switching from any other PNH treatment, **ask your healthcare team when to start taking FABHALTA®**

Engage with your local PNH community

Even though PNH is a rare disease (affecting approximately 1–3 people per million of the population) and you might not know another person with the same condition, you are not alone.

Engaging with your local PNH community can be a valuable way for you to share your experiences with others who understand your disease. It can also provide support and reduce the isolation you may feel.

By engaging with the local PNH community you can:

- Learn ways to effectively manage your symptoms from other PNH patients
- Discuss your emotions and experiences in a safe place with others who know how you feel
- Find comfort and hope from others
- Feel part of a larger community that accepts and values you and your experiences

The Australian support group for PNH patients is the **PNH Support Association Australia (PNHSAA)**. The aim of the PNHSAA is to support PNH patients and their loved ones to manage their condition positively by providing access to accurate information, practical resources and a support network. They also provide opportunities to meet with other patients and caregivers to reduce the isolation often experienced by people with rare diseases. If you would like to find out more about PNHSAA, please scan the QR code or go to:

www.pnhsaa.org.au

Your healthcare team can provide you with some useful contacts, so please ask them about this at your next visit.

Caring for someone who has been prescribed FABHALTA® for PNH

The person you care for has been prescribed a new treatment, FABHALTA®, to prevent haemolysis and control their PNH symptoms. Remember that this treatment can also weaken the body's natural defences against infection.

As a result, people who take FABHALTA® may have an increased risk of serious infections, so it may be useful for you to know the signs and symptoms of these serious infections.

The signs and symptoms of infections are shown on page 15. **If you suspect the person you care for has an infection, you should contact their healthcare team immediately.**

How can I support the person I care for with their new treatment?

PNH is a disease that requires continuous treatment, so it's important that the person you care for sticks to their treatment schedule. This will help them to effectively manage their symptoms on an ongoing basis.

The person you care for needs to take FABHALTA® twice daily, ideally once in the morning and once in the evening. You can play a role in helping them to manage their symptoms and PNH by reminding them to take their treatment as prescribed.

You can set a reminder to alert them for when a dose is due:

- On your phone
- In an app
- In your calendar
- Prepare for changes to their routine e.g., travel and refills

Be an advocate for the person you care for

You can offer to attend appointments with the person you care for. This is a great way to offer support and be an active participant in the appointment by speaking up and asking questions when they speak to their healthcare team.

Here are a few suggestions on how you can advocate for the person you care for:

Bring copies of **reports and test results** to each appointment

Help make sure their **questions are asked and answered**

Ask how you can support **them physically and emotionally**

If you can't attend the visit in person, **ask if you can join via video or phone call**

Take notes during appointments so they can listen more closely while you're writing. You can review the notes together after the appointment

If the healthcare team writes a prescription, make sure you can read the drug name and dosage. Ask the healthcare team what the medication is for, if there are any treatment side effects, and how to manage them

Remember

At times, the person you care for may want to be alone. Try not to take this personally. Part of staying close is giving them space if they need it.

Could the person I care for have side effects from their new treatment?

As with all medicines, there can be unwanted side effects. Not everyone will have them, but as a carer for someone who has been prescribed FABHALTA®, it might be useful for you to recognise some of these side effects.

The most common side effects are:

- Common cold (upper respiratory tract infections)
- Headache
- Diarrhoea

For more side effects, please refer to “Will I have any side effects from my FABHALTA® treatment?” in the “Getting to know your treatment” section of this guide on page 11.

You should seek medical advice immediately if the person you care for experiences severe or persistent side effects.

Is there anything else I can do to support the person I care for?

There are many things you can do to support someone with PNH. No matter how big or small, your care and support can help make a difference to the way they manage their PNH symptoms and improve their daily lives.

Here are a few tips:

- You can help them do things they are too tired to do themselves, like household chores
- You can occasionally drive them to a healthcare team's appointment or help them manage and collect their prescriptions
- You can also provide emotional support by actively listening to what they have to say and engaging directly with it, showing empathy and offering encouragement with their disease management

I don't feel I can do anything practically. Can I offer support in other ways?

Being present is one of the most important things you can do as you care for someone with PNH. They may or may not open up to you about their feelings, but you can let them know that you hear them and acknowledge their emotions.

Here are a few suggestions you may want to try:

- Gentle words
- Touch
- Eye contact
- Silence

This allows the person you care for to understand that you hear them and understand what they are going through. You can't make their PNH go away, but you can be there for them to offer emotional support.

Remember to take care of yourself!

People who act as caregivers are at higher risk of chronic diseases, insomnia, anxiety and depression.

Whilst it's important that you provide care and support for someone with PNH, it's also important that you take steps to protect your own physical and emotional health. If you're not looking after yourself, it will be very difficult for you to look after someone with PNH.

Some helpful tips to encourage your own self-care include:

- Ensuring that you get regular sleep
- Taking regular walks and trying to have some form of movement as part of your daily/weekly routine
- Practising mindfulness techniques such as deep breathing and meditation – this can help relieve stress
- Doing something that you enjoy at least once a week

FABHALTA® summary

Who is it for?

Adults with paroxysmal nocturnal haemoglobinuria (PNH)

How is it taken?

One 200 mg capsule of FABHALTA® is taken by mouth twice daily, with or without food

What type of medicine is it?

FABHALTA® is a Factor B complement inhibitor. It works by stopping part of the complement system, a part of your body's immune system

Very common side effects of treatment (more than 1 in 10 people)

- Common cold (upper respiratory tract infections)
- Headache
- Diarrhoea
- Abdominal pain

Common side effects of treatment (more than 1 in 100 people)

- Decrease in blood platelet count (usually this is mild and short-lived)

Switching to FABHALTA® from any other PNH treatment

- Start your FABHALTA® treatment no later than 1 week after the last dose of eculizumab
- Start your FABHALTA® treatment no later than 6 weeks after the last dose of ravulizumab

Signs of serious infection (that may occur with treatment)

- Fever
- Headache
- Confusion
- Body aches with flu-like symptoms
- Clammy skin
- Eyes sensitive to light

Please refer to page 15 of your treatment guide for more details

Frequently asked questions (FAQs)

How should I report a side effect?

Like with all medicines, FABHALTA® can cause side effects, although not everybody experiences them. If you experience any side effects, talk to your healthcare team immediately. This includes any possible side effects not listed in this guide or the leaflet you will find in your medication package.

Why do I need a patient ID?

The patient ID is part of a controlled access system for FABHALTA®. You will not be able to receive FABHALTA® without your ID. It ensures that FABHALTA® is only dispensed to patients that are fully vaccinated against infections from encapsulated bacteria or protected through antibiotics. This is to minimise your risk of life-threatening bacterial infections. It also protects your privacy from Novartis as all your personal information is anonymised.

What is a patient safety card?

When you are first prescribed FABHALTA®, you will be given a patient safety card.

This card contains important safety information about the risk of infection while taking FABHALTA®, and what to do if you experience certain signs or symptoms.

Keep this card with you at all times during treatment, and for a period of 2 weeks following your last FABHALTA® dose in case of an emergency. You should tell all members of your healthcare team that you are being treated with FABHALTA®.

If you have not received a patient safety card, please contact your healthcare team or nurse.

I've missed a few doses and now I'm worried about haemolysis. Are there any signs and symptoms I should look out for?

While each person may have different symptoms, general signs and symptoms of haemolysis include:

- Tiredness
- Haemoglobin in the urine
- Pain in the stomach (abdomen)
- Shortness of breath
- Blood clots (thrombosis)
- Trouble swallowing
- Erectile dysfunction

If you miss a dose or doses, it's important that you take one dose of FABHALTA® as soon as possible, even if it's shortly before your next dose is due.

I'm trying for a baby. Will this medicine affect my chances of getting pregnant?

The use of FABHALTA® in pregnant women or women planning to become pregnant may be considered following an assessment of the risk and benefits by your healthcare team.

Find the support you need

Beginning a new treatment can be overwhelming. Our website and patient support program are here to provide further information and resources, and to guide you through any questions you may have.

As a patient starting treatment with Fabhalta® (iptacopan) you are invited to enrol in the **replenishEd patient support program**. By joining replenishEd you will have the option to receive:

- Educational emails on managing PNH with oral therapy
- Monthly phone calls with a haematology nurse
- SMS reminders about medication

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Please scan the QR code to enrol in the replenishEd patient support program, or go to: replenished-pnh.com.au

Trouble signing up? Call 1800 290 652

Please scan here to visit our patient website to find more information, and to access electronic copies of your Fabhalta® onboarding materials

Common terms to know

Anaemia

A low level of healthy red blood cells in the blood.

Complement inhibitors

Medicines that reduce the activity of the complement system, a part of the immune system.

Complement system

A part of the immune system that helps fight infection and removes damaged blood cells.

Haemoglobin

A protein in red blood cells that transports oxygen around the body.

Haemoglobinuria

High amounts of haemoglobin in the urine.

Haematuria

Increased occurrence of red blood cells in the urine.

Haemolysis

Destruction of red blood cells.

Haemolytic anaemia

Form of anaemia caused by premature destruction of red blood cells.

Haptoglobin

A protein that is produced in the liver and transports free haemoglobin in the blood.

Factor B

A protein which increases activity of the complement system.

Fatigue

Extreme, persistent exhaustion that does not or hardly improves despite supposedly sufficient rest or sleep.

Meningitis

A serious infection of the protective membranes that surround the brain and spinal cord (meninges), that causes a stiff neck, headache and fever.

Paroxysmal

Seizure-like, occurring intermittently.

Paroxysmal nocturnal haemoglobinuria (PNH)

A rare and serious blood disorder in which red blood cells break apart more easily, leading to anaemia and other complications.

Platelet

A small, colourless, disc-shaped cell fragment, found in large numbers in blood and involved in clotting.

Pneumonia

A serious infection of one or both of the lungs.

Proteins

Large molecules which have various roles in the body, including building and repairing tissues, enzymes and hormones.

Red blood cells

Blood cells that carry oxygen from the lungs to the rest of the body.

Sepsis

Blood poisoning, which has life-threatening consequences; it occurs when a malfunction of the immune system – often triggered by an infection with bacterial pathogens – damages the body's own organs and tissues.

Thrombosis

Clotting of the blood.

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This material is intended only for patients with paroxysmal nocturnal haemoglobinuria (PNH) who have been prescribed FABHALTA®.
This is provided for information/educational purposes only and is not intended to replace discussions with your doctor or care team.

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