

Aspaveli[®] ▼ (pegcetacoplan) patient brochure

▼ This medicine is subject to additional monitoring.
This will allow quick identification of new safety information.
You can help by reporting any side effects you may get.
You can report side effects by contacting your doctor, nurse
or pharmacist or you can report side effects directly via the
Yellow Card Scheme at www.mhra.gov.uk/yellowcard
or search for MHRA Yellow Card in the Google Play or
Apple App Store.

This guide is intended for patients who have been prescribed Aspaveli, and is initiated and funded by Sobti[™]. Please refer to the Aspaveli Patient Information Leaflet (PIL) provided by your doctor or nurse for further information. The PIL is also available at www.medicines.org.uk/emc.

The information provided in this guide is general educational information and does not take the place of professional medical advice. Always follow your doctor or nurse's instructions and talk with them about any questions or problems you have regarding your health and treatment.

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Introduction

This booklet has been given to you because you, or someone in your care, has been prescribed Aspaveli (pegcetacoplan) as a treatment for paroxysmal nocturnal haemoglobinuria (PNH).

This booklet is a guide that explains what PNH is and how it may affect your body. It also explains what Aspaveli is and how it works.

Your PNH specialist, doctor or nurse can answer any specific questions about your diagnosis and treatment management.

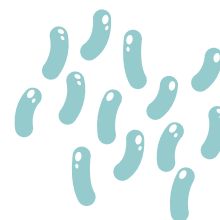
The science behind PNH

PNH is a bone marrow disorder. In PNH, the immune system – the body's natural defence system – attacks and destroys blood cells, particularly red blood cells.

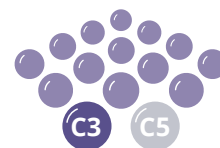
PNH is a rare disease but it can affect anyone of any age, race or gender. No one is born with PNH; it is known as an “acquired disease”, which means it cannot be inherited, cannot be passed on to your children, and it is not contagious. PNH is a lifelong condition.

PNH and the immune system

The immune system is a sophisticated defence network that protects the body from disease and infection. One very complex part of this network is called ‘the complement system’.



The complement system helps your body to destroy foreign attackers, like viruses or bacteria, and gets rid of damaged or dying cells.



There are more than 60 different proteins that play a part in the complement system. Two **key proteins** are known as C3 and C5.



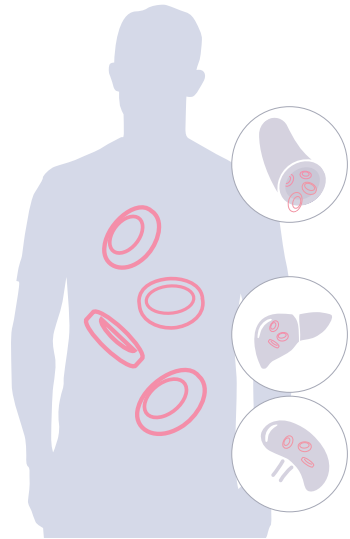
When your immune system detects viruses or bacteria, it sets off a chain reaction called the ‘complement cascade’. Like dominoes, one reaction leads to another, and C3 and C5 are ‘activated’ as part of this cascade.



When C3 and C5 are activated, they attack viruses and bacteria and break them apart. This is normal, but in people with PNH, another reaction happens – PNH red blood cells also get broken apart. This breakdown of red blood cells is known as ‘haemolysis’.

Intravascular haemolysis (IVH) and extravascular haemolysis (EVH)

Haemolysis (the breakdown of red blood cells) can happen either inside your blood vessels, or in other parts of your body.



Intravascular haemolysis (IVH) is when the breakdown of PNH red blood cells happens **inside** the blood vessels.

Extravascular haemolysis (EVH) is when the breakdown of PNH red blood cells happens **outside** of the blood vessels. For example, in the liver or spleen.

Both types of haemolysis may be responsible for ongoing PNH symptoms:



Anaemia



Fatigue



Abdominal pain
and/or chest pain



Trouble
swallowing



'Brain fog'



Headaches



Shortness of
breath



Issues with
intimacy or
sexual health



Feelings of
weakness



Loss of appetite



Blood in
urine

Aspaveli can treat both types of haemolysis

Until recently, only C5 inhibitors have been available for people with PNH. C5 inhibitors work to prevent red blood cell breakdown inside blood vessels (IVH), but they have no effect on the breakdown of red blood cells in other parts of the body (EVH). This means red blood cells continue to be broken down, and as a result, you may continue to experience PNH symptoms despite being on treatment.

Aspaveli is a C3 inhibitor, which means it can target both types of red blood cell breakdown (EVH and IVH). You have been prescribed Aspaveli because your symptoms have not been controlled well enough by C5 inhibitors, and your doctor believes that it may be beneficial for you to try a C3 inhibitor.

About Aspaveli



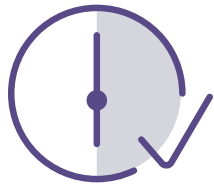
Aspaveli is a medicine that contains the active substance pegcetacoplan. It comes in the form of a liquid which is supplied in a vial.

Aspaveli should be administered twice-weekly by subcutaneous (under the skin) infusion, using one vial per infusion. In some circumstances, your doctor or nurse might change the frequency of your dose.

This means a needle is inserted into the fatty layer of tissue just under the skin to deliver the medicine. Unlike an intravenous injection, the needle does not go into a vein.

To support your infusion, you will be provided with an infusion pump, which will help deliver Aspaveli into your body at a controlled rate.

You can infuse Aspaveli into your abdomen, thighs, hips, or upper arm. It is recommended that you change the infusion site for each infusion, rather than always using the same site.



The typical infusion time is approximately 30 minutes when using two infusion sites, or 60 minutes when using one infusion site.



Your first Aspaveli infusion/s will be administered in the hospital, where you and/or your caregiver will receive training from your doctor or nurse on how to set up your infusion, and how to use the infusion pump. You will also be given additional support and training at home, and will then be able to self-administer Aspaveli at home, or at another location that is suitable and convenient for you.



Your Aspaveli will be delivered to you at home along with the other items that you need to administer your infusions. This will include a needle disposal bin, which will then be collected from you when you receive your next delivery.



A detailed self-infusion guide and a self-administration video will also be provided to support you with your infusions.

Please refer to the Aspaveli Patient Information Leaflet (PIL) provided by your doctor or nurse for further information. The PIL is also available at www.medicines.org.uk/emc.

Frequently Asked Questions

How should Aspaveli be stored?

Aspaveli can be used until the expiration date that is printed on the carton. Do not use the vial if the expiration date has passed.

Aspaveli should be stored in the refrigerator. It should be kept between 2°C to 8°C (35.6°F to 46.4°F), and kept in the original carton to protect it from light. It may be helpful to keep a thermometer in your refrigerator. Do not allow Aspaveli to freeze.

Do not store Aspaveli at the back of the refrigerator to avoid freezing. Store Aspaveli in the middle of the refrigerator on one of the shelves, not in the door, to minimise changes in temperature. Do not use Aspaveli if the liquid in the vial is frozen. If there is a power cut, or your refrigerator breaks, contact your homecare provider.

If you have any questions on storage of Aspaveli, contact your homecare provider or your doctor or nurse.

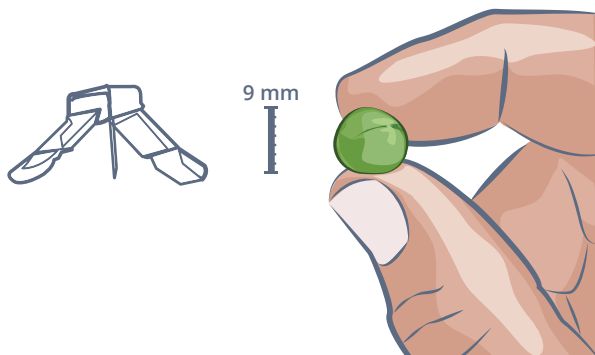
What should I do if my vials of Aspaveli freeze?

Aspaveli should not be frozen. Do not use Aspaveli if the contents of the vial become frozen at any stage. To avoid freezing, do not store Aspaveli at the back of the refrigerator.

If a vial does become frozen, contact your homecare provider for advice.

How big is the needle that I will use to administer Aspaveli?

Typically 26-gauge needles are used for the subcutaneous infusion. The needle is typically around 9 mm long, which is only about the length of a pea.



Will there be any discomfort from a subcutaneous infusion?

Subcutaneous infusions are considered to cause less discomfort than intravenous infusions.

'Subcutaneous' means the needle goes just under the skin. Some people may experience some discomfort at the needle site or during the infusion, however, it is mostly tolerable.

What should I do if I experience discomfort during the infusion or see a mark or rash on my skin at my infusion site?

If you experience any discomfort or are concerned during your infusion, contact and report it to your doctor, nurse or homecare provider. The Aspaveli Patient Information Leaflet explains the expected side effects for Aspaveli.

You should report side effects by contacting your doctor, nurse or pharmacist or you can report side effects directly via the Yellow Card Scheme at www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. You can also report side effects to Sobi, please email drugsafety@sobi.com or call +44 (0) 800 111 4754.

What if my infusion is taking less or more time than expected?

The approximate infusion time, if you are using two infusion sites, is 30 minutes. If you are using one infusion site, it is 60 minutes. These are approximate infusion times and may be different for each person, so do not worry if your infusion is a bit faster or slower. If you have any questions about your infusion time contact your homecare provider or your doctor or nurse.

What should I do if I miss an infusion of Aspaveli?

If you miss a dose of Aspaveli, it should be taken as soon as possible, and you should let your doctor or nurse know. You should then take the next dose as regularly planned.

It is recommended that you keep a record of the doses you administer and be sure to take the record with you each time you visit your doctor or nurse because they may ask to see it.

Do I have to administer each infusion at the same time of day?

You may find it helpful to take your infusion at the same time of day to establish a routine, however it is not a problem if you change the time that you administer your infusion as long as it is on the same day as your regular schedule.

What should I do if I break a vial of Aspaveli or any of my infusion supplies, or if there are problems with my infusion pump?

If you break a vial of Aspaveli or any of your infusion supplies or have problems with or questions about your infusion pump, contact and report it to your doctor, nurse or homecare provider to let them know.

You should report product complaints (quality issues or defects with the product, pump and infusion supplies) by contacting your doctor, nurse or homecare provider. You can also report product complaints to Sobi, please email medical.info.uk@sobi.com or call +44 (0) 800 111 4754.

What you need to know before starting your treatment with Aspaveli

Risk of serious infections

The use of pegcetacoplan targets the complement system, which is part of the body's defences against infection. As such, the use of this medicine increases your risk of infections, including those caused by the so-called 'encapsulated bacteria', such as *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae*. These are severe bacterial infections affecting your nose, throat and lungs or the linings of the brain and can spread throughout the blood and body. Serious bacterial infections may quickly become life-threatening and cause death if not recognised and treated early.

Call your doctor or nurse or seek emergency medical help right away if you have any of these signs and symptoms of a serious infection:

- Headache and a fever
- Fever and a rash
- Fever with or without shivers or chills
- Shortness of breath
- High heart rate
- Clammy skin
- Headache with a stiff neck or stiff back
- Headache with nausea (feeling sick) or vomiting
- Eyes sensitive to light
- Muscle aches with flu-like symptoms
- Confusion
- Extreme pain or discomfort

Risk of allergic reactions

Allergic reactions may occur in some patients receiving treatment with pegcetacoplan.

Immediately stop pegcetacoplan infusion if you develop any of these signs and symptoms of an allergic reaction and seek immediate medical attention:

- Difficulty breathing
- Chest pain or chest tightness
- Feeling dizzy/faint
- Severe itching of the skin or raised lumps on the skin
- Swelling of the face, lips, tongue and/or throat, which may cause difficulty in swallowing or collapse

Prophylactic vaccinations or antibiotic treatment

Vaccines against bacteria lower the risk of getting serious infections. However, vaccines do not prevent all serious infections.

Your doctor will ensure that you receive vaccination against the bacteria *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* if you have not had these vaccines in the past.

If you have had these vaccines in the past, you might still need additional vaccinations before starting treatment with pegcetacoplan. Your doctor will advise if you need additional vaccinations.

These vaccinations should be given at least 2 weeks before beginning pegcetacoplan therapy.

If you cannot be vaccinated 2 weeks before beginning pegcetacoplan therapy, your doctor will prescribe antibiotics (medications to treat bacterial infections) to reduce the risk of infection, for 2 weeks after you have been vaccinated. Pegcetacoplan will only be supplied if your doctor or pharmacist submits proof that you are receiving the required vaccinations or antibiotics.

Your doctor or pharmacist will receive annual vaccination reminders and will contact you in case you need revaccination. It is important that your vaccinations are up to date, so speak with your doctor or nurse about this. You should also be aware that vaccines will reduce the risk of serious infections, but do not prevent all serious infections.

What you need to know...continued

Risk of destruction of red blood cells (haemolysis) after drug discontinuation

- Do not stop treatment with pegcetacoplan without discussing this beforehand with your doctor or nurse.
- It is very important to make sure that you do not miss or postpone any scheduled treatments. If you stop taking the medicine suddenly, you may be at risk of making your symptoms worse.
- If pegcetacoplan treatment is stopped completely, postponed, or if treatments are missed, there is a risk that haemolysis could occur. Haemolysis is when red blood cells, which carry oxygen through your body, break apart. Haemolysis is connected to various symptoms of PNH, such as:

- Tiredness (fatigue)
- Dark urine (haemoglobinuria)
- Tummy (abdominal) pain
- Breathlessness
- Formation of blood clots (thrombosis)
- Difficulty in swallowing
- Erectile dysfunction

- Seek immediate medical attention if you notice any signs or symptoms of haemolysis.

Reporting side effects

Reporting side effects of your treatment is important as it allows collection of more information about the safety of Aspaveli. If you experience any side effects (this also includes any possible side effects not listed in the Patient Information Leaflet), in particular serious infections with encapsulated bacteria, severe hypersensitivity reactions, or haemolysis after drug discontinuation, inform your doctor, nurse or pharmacist.

You can also report side effects directly via the Yellow Card Scheme at www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. You can also report side effects to Sobi, please email drugsafety@sobi.com or call +44 (0) 800 111 4754.

By reporting side effects, you can help provide more information on the safety of this medicine.

Further Support Available

PNH Support

PNH Support is a registered charity (No 1161518) run by PNH patients and carers as a peer-to-peer support organisation for people living with PNH (and their carers and family members) in England, Wales and Northern Ireland. Our activities include: facilitating PNH patients and carers to share their experiences and support one another; advocating on behalf of PNH patients and carers with stakeholders; and providing or signposting to further information.

How to get in touch:

Email: contact@pnhuk.org

PNH Scotland

PNH Scotland is a charity dedicated to supporting anyone affected by PNH. Our main aim is to raise awareness of the condition and advance the education of patients, their families and interested health professionals. In particular, we aim to ensure all patients receive access to the treatment they require, provide up to date information and act as a point of contact for anyone needing help and advice.

How to get in touch:

Email: info@pnhscotland.org.uk

Tel: +44 (0) 7967 118762

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