

Hydroxycarbamide (Hydroxyurea) Treatment for Sickle Cell Disorder for Adults



Information for patients, families and carers

Haematology - Haemoglobinopathy
Coordinating Centre



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What is Hydroxycarbamide?

Hydroxycarbamide is a daily, oral medication given to people with sickle cell disorder to reduce short and long term complications of the condition.

It has been used for many decades in the UK, Europe and USA and we have a lot of information about how it works and how it benefits people.

It can be taken as liquid, tablets or capsules.

What is Hydroxycarbamide used for and who should take it?

Hydroxycarbamide is used to treat sickle cell disorder and its complications. It reduces the frequency and severity of sickle cell crises including painful crisis and acute chest syndrome. It also reduces the need for blood transfusion and the risk of needing hospital admission.

Hydroxycarbamide provides some protection against long-term organ damage (for example in the kidneys and brain) and on average will increase how long someone will live when taken over a long time.

For people with the most common types of sickle cell disorder (HbSS), this medication is now recommended for everyone from the age of 9 months, even for people who have not yet experienced any serious complications. This is because of the long term benefits including improved life expectancy. It is also appropriate for some people with other types of sickle cell disease, such as HbSC, especially if they have problems related to the sickle cell.

How does Hydroxycarbamide work?

In sickle cell disorder, red blood cells change from being healthy, round and flexible and instead turn into rigid and spiky 'sickle' shapes. These sickle cells stick to the blood vessel walls, cause blockages to the blood vessels and restrict blood flow. They also break down quickly in the circulation, causing anaemia.

Hydroxycarbamide works in a number of different beneficial ways to keep the red blood cells healthy and stop them turning into sickle cells:

- Hydroxycarbamide increases the amount of foetal (baby) haemoglobin (HbF). HbF is the type of haemoglobin which you were born with, and which normally mostly disappears by 6 months of age. When you take Hydroxycarbamide, your body reverts back to making more HbF which helps to stop the red blood cells turning into sickle shapes.
- Hydroxycarbamide helps the red blood cells to stay hydrated, which makes it more difficult for them to turn into a sickle shape.
- Hydroxycarbamide helps to stop the red blood cells sticking to the lining of the blood vessels and to other blood cells, which can bring on a crisis.
- Hydroxycarbamide reduces the number of white blood cells and platelets in the circulation. They are often higher than normal in people with sickle cell disorder, and bringing their numbers down helps to prevent sickle crises by making the blood less 'sticky'.
- Hydroxycarbamide also increases the levels of a substance called nitric oxide in the blood which helps with blood flow through the tissues of the body.

Together, these effects combine to help to stop red blood cells becoming sickle cells, reducing how often people have a sickle crisis. Because the red blood cells are healthier this also reduces anaemia and improves fatigue.

What are the benefits of using hydroxycarbamide?

We know from the evidence provided by large clinical trials that hydroxycarbamide provides the following benefits:

1. Helps to reduce the number of sickle cell crises
2. Reduces the severity of pain
3. Reduces the risk of acute chest crisis
4. Reduces the need for blood transfusions
5. Reduces the need for hospital admission
6. Help to prevent damage to the spleen, kidneys, brain and other organs
7. Helps people with sickle cell disorder to live longer and healthier lives

Starting and monitoring of hydroxycarbamide

Hydroxycarbamide is usually started at a low dose based on your weight, and this will be increased by your doctor or specialist nurse until you reach what is known as your 'maximum tolerated dose'. This is the dose which gives you the best level of protection without causing any problems or side effects.

When you first start hydroxycarbamide you will need a repeat blood test in 2 weeks to make sure that it has not reduced your blood counts by too much.

You will also need a repeat blood test 2 weeks after any increase in your dose. Once you are on a stable dose of hydroxycarbamide, you will have blood tests approximately every 3 months, which can often be coordinated with your clinic appointments.

If you do not live near your specialist centre, or find it difficult to get to the hospital for your blood tests, speak to the Haemoglobinopathy team about whether you can have your blood tests at a more local hospital or at your GP surgery instead.

Possible side effects of hydroxycarbamide

All medications have possible side effects but the risk of having any serious side effects from hydroxycarbamide are low and for most people the benefits are much greater than any potential side effects. The long-term safety of hydroxycarbamide is very good.

There are a few things to be aware of though before starting hydroxycarbamide:

- Hydroxycarbamide can cause a fall in your white blood cells, platelets or haemoglobin (although usually haemoglobin is better on hydroxycarbamide). This is the reason you start on a low dose and have regular blood tests during your treatment. It is important that you attend to have these checks.
- Lowering the white blood cells can lower your resistance to infection. If at any time you develop a fever (temperature above 38°C), sore throat, or if you suddenly feel unwell, you should contact your specialist team for review immediately. This does not happen often, but if it does you may need treatment with strong antibiotics.
- Hydroxycarbamide can reduce the number of platelets, which help your blood to clot. If your platelets are too low, you can develop bruising and bleeding – if you notice these symptoms, it is important to let your haemoglobinopathy team know straight away.
- Usually your haemoglobin (red blood cell level) will improve when you are taking hydroxycarbamide, but occasionally it can fall if the dose is too high. If it falls you might feel more tired than usual.

Other side effects

These are less common and include the following:

- nausea
- diarrhoea
- darkening of skin or nails
- temporary hair thinning.

Sperm count

Sperm count count is lower in men while they are taking hydroxycarbamide, but this effect is expected to wear off once a man stops taking the hydroxycarbamide. We know that sickle cell disorder itself causes lower sperm counts, and therefore taking hydroxycarbamide might actually help to protect fertility in boys and men who take this medication, as it helps to reduce damage caused to the testicles by the sickle cell.

Pregnancy

- Women are advised not to get pregnant whilst taking hydroxycarbamide and to stop taking the medicine three months before planning a pregnancy. Women cannot restart hydroxycarbamide until after the pregnancy, and after they have finished breastfeeding.
- Men are also advised to stop taking hydroxycarbamide for three months before trying to conceive a child. Therefore men or women taking hydroxycarbamide should use effective contraception.
- Men and older boys are recommended to store a sperm sample before they start taking hydroxycarbamide – this means that the frozen sperm can be used to conceive a pregnancy, and in this case they do not have to stop taking the hydroxycarbamide to have a child.

How long will it take before I notice an improvement?

Everyone is different, most people respond well to hydroxycarbamide and should notice an improvement within 3-6 months. Some people feel the benefit more quickly, within a week or two. Hydroxycarbamide has to be taken every day, it will not work if only taken every now and again. It is not a painkiller, or an immediate treatment for complications - it works over the longer term when taken regularly.

If you have any further questions please speak to your specialist nurse or doctor for further information.

Please use the QR code or link below to take you to the Patient Information Leaflet (PIL) section of the North East and Yorkshire Haemoglobinopathy Co-ordinating Centre Website

Patient Information - NEYHCC



Further information

The Sickle Cell Society provides advice, information and support to people with SCD and their families, and raises funds for the education of carers and health professionals, phone: 020 8961 7795, website: www.sicklecellsociety.org



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