

Patient information

Sickle Cell Disorders Using Hydroxycarbamide (Hydroxyurea)

Haematology Department

This information sheet has been given to you to help answer some of the questions you may have about taking Hydroxycarbamide for the management of sickle cell disease. It contains information on the benefits and risks of taking hydroxycarbamide, how to take and store it, and how it works, including the changes it causes in the blood.

What is hydroxycarbamide?

Hydroxycarbamide is a medicine taken in capsule form. It causes changes in the blood, which reduce the frequency and severity of sickle cell crises and the need for transfusion in some patients with sickle cell disease. It may also be called hydroxyurea.

How does hydroxycarbamide work?

Sickle cell disease is a condition where the patient inherits a different form of haemoglobin (the chemical in red blood cells that carries oxygen). This different haemoglobin causes the red blood cells to sickle (change from a soft, round disc into a hard sickle or banana shape).

Hydroxycarbamide increases the amount of fetal or 'baby' haemoglobin (HbF) in red blood cells. Fetal haemoglobin is the main haemoglobin found in babies before birth but its production tails off after birth. Most adults only have a very small amount of HbF present in their red cells (less than 1%).

Hydroxycarbamide switches part of this production back on and as a result, the level of HbF starts to rise again. HbF prevents blood cells from sickling.

Giving hydroxycarbamide to sickle cell patients will reduce the severity of sickle cell disease in some patients by:

- increasing the time that red blood cells survive in the body, which in turn reduces both anaemia (low haemoglobin) and jaundice (yellow eyes)
- making the red blood cells and the lining of the blood vessels less sticky, so the blood vessels are less likely to be blocked by clumps of sickle cells
- reducing the number of white blood cells, especially the cells called neutrophils. Neutrophils are important in fighting infection in the body, but the high neutrophil counts often seen in people with sickle cell disease can cause inflammation and trigger sickle cell crises and other complications
- reducing the number of the blood cells involved in clotting (platelets).

Will hydroxycarbamide work for me?

Hydroxycarbamide does not work for all patients with sickle cell disease, but most feel an improvement in their health within three months of starting treatment.

You will need to discuss the treatment with your doctor or sickle cell nurse in the sickle cell clinic.

Hydroxycarbamide is often recommended if:

- you have moderately severe or severe sickle cell disease
- you have had two or more acute hospital admissions for sickle cell crisis in the last 12 months
- you have experienced acute sickle chest syndrome and needed a blood transfusion or admission to the high dependency unit; and/or
- you have had a stroke and are no longer tolerating an exchange blood transfusion programme.

Do I have a choice about taking hydroxycarbamide?

It is entirely your choice whether to start taking hydroxycarbamide and for how long you take it.

If you agree to start this treatment, your doctor will ask you to sign a consent form.

This confirms that you understand why hydroxycarbamide has been recommended for you, and the risks and benefits of the treatment.

If, after discussing everything with your haematologist you still feel uncertain, you do not have to take it.

It has been recommended as a treatment option for you, on the basis of your particular diagnosis of sickle cell disease, but this does not mean you have to choose this treatment. We will support you whatever decision you make about your treatment as we want to keep you as healthy as possible.

If you do start taking hydroxycarbamide but then decide to stop, we strongly recommend that you discuss this with your haematologist. When you stop taking it, your other treatment will continue as it did before you started taking hydroxycarbamide.

Are there any alternative treatments available?

Hydroxycarbamide is an additional option to the treatment you are currently receiving. Your medical team will always be happy to discuss any treatment options.

What will happen if I decide not to have treatment?

If you decide not to have hydroxycarbamide your treatment will remain as it currently is. Hydroxycarbamide has been offered as it is believed there may be some benefits to you as listed below.

What are the benefits of taking hydroxycarbamide?

- It should reduce the number and severity of crises.
- It should reduce the risk of sickle cell chest syndrome (chest crisis).
- You will need fewer blood transfusions.
- It can increase your life expectancy.
- It can improve quality of life.

Hydroxycarbamide does not reduce the frequency of, or prevent, the following sickle cell complications:

- leg ulcers
- bone damage (avascular necrosis)
- infections.

The role of hydroxycarbamide in preventing stroke is not clear and is still being researched. It is also uncertain if hydroxycarbamide is effective in preventing painful erections of the penis (priapism).

Hydroxycarbamide is not a painkiller but it may help pain by reducing the number of times you experience painful crises.

What are the side-effects of hydroxycarbamide?

Hydroxycarbamide affects the bone marrow, where all your blood cells are formed.

This may occasionally lead to:

- anaemia (a fall in the haemoglobin level);
- a low white cell count, which may increase the likelihood of infection if they are very low; or
- a low platelet count, which may increase the likelihood of bleeding and bruising if they are very low.

The signs of a bone marrow problem are increased tiredness, sore throat, infections or high temperatures and increased bleeding and bruising. If you notice any of these you should stop the hydroxycarbamide, contact your haematologist or clinical nurse specialist and attend hospital for an urgent blood test.

It is important that you seek urgent medical advice as soon as you recognise that you have these symptoms.

Less common side-effects include:

- nausea and vomiting
- loss of appetite
- sore mouth and mouth ulcers
- diarrhoea

- thinning of the hair
- gout (and you should tell your doctor if you have had gout before)
- skin rash
- darkening of the colour of your skin and nails.

These side-effects are usually mild and can be treated simply and effectively by your doctor.

Is there a risk of developing cancer by taking hydroxycarbamide?

Hydroxycarbamide is a very mild form of chemotherapy. It has been used in a range of blood and bone marrow diseases that can change into leukaemia over time. In these patients, who already have an abnormal bone marrow and an increased risk of developing cancer, hydroxycarbamide therapy has been linked to an increased risk of cancer.

Because of this, hydroxycarbamide was at first only given to patients with sickle cell disease who were extremely unwell, with few other alternatives. It was then given to more and more adult patients and finally to children.

Hydroxycarbamide has now been used for more than 15 years in thousands of patients with sickle cell disease across the world. Over that time, only a handful of cancer cases have been reported, which is no more than we would expect in the normal population. Long-term data shows there is probably no increased risk of developing cancer if you take hydroxycarbamide.

On the other hand, comparisons have been made over a ten year period between people with sickle cell disease taking the drug and those who do not. More people who took the hydroxycarbamide were alive at the end of the ten years, than those who did not.

Taking hydroxycarbamide

It is very important that hydroxycarbamide dosage is prescribed and controlled by a specialist doctor within the sickle cell clinic. We will prescribe the hydroxycarbamide in the clinic and check your blood count regularly all the time that we are prescribing it.

Your family doctor (GP) will only prescribe it if they have a special arrangement with the clinic (if you live a long way from the clinic or find it difficult to get to the clinic).

How much hydroxycarbamide will I have to take?

Hydroxycarbamide will be given to you in a capsule form and is taken by mouth. It can be taken with food or on an empty stomach. Each capsule contains 500mg of hydroxycarbamide.

Treatment starts at a low dose and is slowly built up if your body tolerates it well. Typical doses for adult sickle cell patients range from one to four capsules each day. The dose you are given will depend on your body weight, kidney function and individual tolerance. Some patients can only tolerate lower doses.

The dose is changed depending on your blood counts and your doctors are aiming to reach the highest possible dose that your bone marrow will tolerate. The exact dose is less important than the effect it is having on your blood counts and the HbF level in your red blood cells.

If you take more than prescribed, this can stop your bone marrow working normally which can cause serious health problems.

Do I have to take hydroxycarbamide all the time?

For hydroxycarbamide to work properly you have to take it every day. However, if you have an infection and temperature you should stop taking it and seek medical advice.

During office hours you can contact the Sickle Cell Team at the Royal Liverpool University Hospital on the numbers at the end of this leaflet. At other times you will need to attend your local Emergency Department (A&E) Department. Normally, your haematologist will recommend that you restart hydroxycarbamide when your blood count has been checked and is normal.

What if I forget to take a dose of hydroxycarbamide?

Take your normal dose the next day, but **do not take a double dose**. Also, tell your haematologist the next time you go to clinic how many times you have missed a dose. If you are sick (vomit) just after taking your capsules, ask your haematologist for advice.

How long should I take it for?

You should continue to take hydroxycarbamide, as advised, unless your haematologist or another doctor tells you not to. If you want to stop taking it, please talk to your haematologist beforehand.

If after a trial period of several months there has been no medical benefit to your sickle cell disease, despite taking the right dose of hydroxycarbamide, your doctors will advise you to stop taking the drug.

How will my health be monitored?

When you start taking hydroxycarbamide, you will need to see the haematologists and have blood tests (blood counts, HbF level, liver and kidney function) regularly. At first, or if the dose is changed, you will be seen at least every four weeks.

As soon as your blood measures are stable on the hydroxycarbamide you will be seen every eight to twelve weeks.

Your dose may be reduced if your blood count falls or if your liver and kidney blood tests show any changes. We will not be able to prescribe hydroxycarbamide if you are not able to come for regular blood tests, as we need to be able to monitor you.

When will I notice any improvement in my sickle cell disease?

It usually takes at least three months before sickle cell patients can tell if the hydroxycarbamide is working, so it is important to give the medication time to work and for you to take it as prescribed. Hydroxycarbamide will not work if you only take it every now and again or only when you are ill or in pain.

Looking after your hydroxycarbamide

- Keep the capsules in a cool dry place.
- Keep the capsules out of the reach of children. They can be harmful if children swallow them.
- If you stop taking hydroxycarbamide for any reason, please return your capsules to the

- pharmacy.

Do not throw them in the bin or flush them down the toilet.

Can I take other medications whilst I am taking hydroxycarbamide?

You should tell your doctor about all the other medications you are taking when you start using hydroxycarbamide. Occasionally the dosage will need to be changed because of your other medications. You should also tell your doctor if you start any new medications. This includes medicines bought at a pharmacy or elsewhere.

Is there a risk to my fertility or pregnancy whilst taking hydroxycarbamide?

For men:

Hydroxycarbamide does not stop men from having normal erections. It does not prevent you from getting someone pregnant or fathering children. However, a man's sperm count may be reduced and there is a possibility of abnormal sperm being produced while taking hydroxycarbamide.

This will usually return to normal after the hydroxycarbamide has been stopped for two to three months.

We recommend that you continue to use contraception whilst using hydroxycarbamide and that you stop using hydroxycarbamide at least three months before trying for a baby.

In one or two cases the sperm count has not returned to normal, even when hydroxycarbamide was stopped for over a year. We therefore recommend that a sperm sample is checked before you start hydroxycarbamide and that a sperm sample is stored. Your haematologist can organise for you to go and have this done.

For women:

Women should not become pregnant while they or their partner is taking hydroxycarbamide, as research suggests that fetal abnormalities may occur. You should use an effective form of contraception to avoid pregnancy. You can ask your general practitioner (GP), practice nurse or the sickle cell team for further advice.

If you become pregnant you must stop the hydroxycarbamide and inform your doctor immediately.

What if my partner and I want to have a baby?

If you or your partner is taking hydroxycarbamide, you should stop taking it at least three months before trying to conceive. This will reduce the risk to your baby. **You must discuss this with your haematologist first.**

Can I breast feed my baby while taking hydroxycarbamide?

You **should not** breast feed while taking hydroxycarbamide, as small amounts of the drug may be present in your breast milk. You can either breast feed and not take hydroxycarbamide or bottle feed your baby and start taking hydroxycarbamide again. You should discuss this with your haematologist before making a decision.

Feedback

Your feedback is important to us and helps us influence care in the future

Following your discharge from hospital or attendance at your outpatient appointment you will receive a text asking if you would recommend our service to others. Please take the time to text back, you will not be charged for the text and can opt out at any point. Your co-operation is greatly appreciated.

Further information

If you have any questions or concerns, please do not hesitate to speak with the haematologists (blood specialists) or sickle cell specialist nurse

Specialist Haemoglobinopathy Team:

**Consultant Haematologist
Lead Clinical Nurse Specialist
Clinical Nurse Specialist x 2
Haematology Specialist Registrar (Rotational Position)
Clinical Psychologist**

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