

Hydroxycarbamide (hydroxyurea) for children with sickle cell disease



Information for patients

This leaflet explains how a drug called hydroxycarbamide works, and the benefits and risks of taking it. It contains important information to help you decide if your child should take it. If you have any questions or concerns, please speak to your child's haematology doctor.

Confirming your identity

Before you have a treatment or procedure, our staff will ask you your **name** and **date of birth** and check your **ID band**. If you don't have an ID band we will also ask you to confirm your address.

If we don't ask these questions, then please ask us to check.

Ensuring your safety is our primary concern.

What is hydroxycarbamide?

Hydroxycarbamide (hydroxyurea) is a drug used to treat sickle cell disease. It has been used for this purpose for more than 20 years. It has proven effects and helps to reduce the risk of certain complications.

There is good evidence that it works well in children when started at a very young age, with one study showing benefits from the age of 9 months.

What are the benefits of my child taking hydroxycarbamide?

The benefits of taking this drug include:

- reduced episodes of severe pain
- reduced severity of pain
- reduced number of attacks of acute chest syndrome
- reduced admissions to hospital
- reduced need for blood transfusions
- possible prevention of damage to spleen, kidneys and other organs if taken from an early age
- reduced damage to arteries in the brain and protection against stroke

How does hydroxycarbamide work?

Normal red blood cells are a disc shape. If a person has sickle cell disease, their red blood cells change to a sickle shape (similar to a crescent). This is known as sickling.

Normal red blood cells are also flexible and move easily through blood vessels. In sickle cell disease, the red blood cells are stiff and sticky. They stick to other blood cells and vessels. They block blood vessels and reduce the oxygen supply to tissues, causing damage



and pain. They are also destroyed quickly, causing anaemia and jaundice (yellowness of the white part of the eye).

Hydroxycarbamide corrects some of the changes in the blood caused by sickle cell. It helps most people who take it for sickle cell.

Hydroxycarbamide works in a number of ways. These include:

- Increasing fetal (baby) haemoglobin in red blood cells. This is the main type of haemoglobin babies are born with. Usually, the amount of fetal haemoglobin in blood falls to low levels in the first two years of life because our bodies begin to switch off its production when we are born. This type of haemoglobin does not cause sickling. So increasing the amount of fetal haemoglobin in the body can help.
- Reducing the breakdown of red blood cells. This improves anaemia and haemoglobin levels and reduces the jaundice.
- Making the red blood cells and the lining of the blood vessels less sticky. This means the red cells stick less to the lining of the blood vessels and are less likely to block blood flow.
- Reducing the number of white cells. They play a part in sickle cell complications, so having fewer to a degree is helpful.

How is hydroxycarbamide taken?

Hydroxycarbamide is taken by mouth, usually once a day. It is usually a tablet but can be given as a liquid in younger children. It is started at a dose based on your child's weight and then gradually increased.

Some people notice an improvement on a low dose, but others need a higher dose to benefit. The dose is adjusted gradually and blood tests are checked regularly, usually every two to three months.

We will prescribe your child's hydroxycarbamide in the clinic and the hospital pharmacy will supply it. GPs do not usually prescribe this



medication, because a specialist needs to monitor your child and the dosage.

How to take hydroxycarbamide:

- It is very important that your child takes the correct, prescribed dose.
- If they miss a dose, take it as usual the next day. Do not take a double dose. It is important to tell your doctor if they miss any doses.
- Take it with food or on an empty stomach.
- Keep the capsules in a cool, dry place.
- Make sure you have enough supply and do not run out. Get a repeat prescription when you come to the sickle cell clinic.
- Continue to give it to your child as advised, unless your child's haematologist or another doctor tells you not to.
- If your child tends to forget to take medicine, think of ways to help them remember. They could set an alarm on a mobile phone or place a note on the fridge door. Developing a routine and taking the medication at the same time of the day also helps.

What will my child or I notice after taking the medicine?

The effects of hydroxycarbamide are not immediate, although some people report feeling better quite soon after starting.

Most people find that they have a generally better quality of life on hydroxycarbamide with fewer episodes of pain, more energy, fewer emergency visits to the hospital or GP and more time at school and doing enjoyable things.

To be effective, the medicine must be taken every day. We usually suggest a trial period of at least six months to decide how well it is working.



What are the risks and side effects?

Hydroxycarbamide has been extensively tested in babies, children and adults. It is considered a safe drug. But like most drugs, it can have side effects. For most people, the benefits of having this treatment are greater than any possible side effects. Most people do not have any problems with it.

The main risks and side effects include:

- Rashes. Some people notice a faint skin rash or darkening of the skin and nails. This is not harmful and goes away when the hydroxycarbamide is stopped.
- Sickness. Occasionally people feel sick or unwell when taking hydroxycarbamide. This is rare and not usually severe.
- Blood count. Hydroxycarbamide reduces the white cell count in the blood. This is a good thing but if the white blood cells get too low, there is an increased risk of infection and the hydroxycarbamide has to be stopped temporarily to allow the count to increase again. It is important that your child has regular blood tests for this reason.

In older boys, hydroxycarbamide reduces the sperm count while it is being taken. This should recover when hydroxycarbamide is stopped. There is a very small possibility that the sperm count might remain low, although this might not affect fertility. The advice to teenage boys is to provide a sperm sample that can be stored for future use if there is difficulty fathering a child.

Long-term side effects

Hydroxycarbamide is a form of mild chemotherapy and the beneficial effects in sickle cell disease are a result of its action on the bone marrow. We all have a low risk of developing leukaemia or other cancer during our lifetime and some forms of



chemotherapy seem to increase that risk. There is no evidence that hydroxycarbamide itself can cause leukaemia in patients with sickle cell disease. There is however a risk of damage to the bone marrow if taken over long periods of time.

Are there any alternatives?

Hydroxycarbamide is effective in more than 80% of people who take it and is currently the best option for modifying the severity of sickle cell disease in some patients. Regular blood transfusions are given to some children as treatment for sickle cell disease, although this is a more difficult treatment with more complications.

Newer drugs are being developed but none of these can be prescribed routinely in the UK at the moment. We are happy to discuss these with you in clinic. Bone marrow transplantation is currently the only cure for sickle cell disease.

Who can I contact with queries and concerns?

The Sickle Cell Team are always available to answer your questions. You can contact us through the paediatric haematology office on 020 3299 3773.

You can also contact one of the Sickle Cell Specialist Nurses on 020 3299 4752 or 020 32991916.

Sharing your information

We have teamed up with Guy's and St Thomas' Hospitals in a partnership known as King's Health Partners Academic Health Sciences Centre. We are working together to give our patients the best possible care, so you might find we invite you for appointments at Guy's or St Thomas'. To make sure everyone you meet always has the most up-to-date information about your health, we may share information about you between the hospitals.



Care provided by students

We provide clinical training where our students get practical experience by treating patients. Please tell your doctor or nurse if you do not want students to be involved in your care. Your treatment will not be affected by your decision.

PALS

The Patient Advice and Liaison Service (PALS) is a service that offers support, information and assistance to patients, relatives and visitors. They can also provide help and advice if you have a concern or complaint that staff have not been able to resolve for you. The PALS office is located on the ground floor of the Hambleden Wing, near the main entrance on Bessemer Road - staff will be happy to direct you.

PALS at King's College Hospital, Denmark Hill, London SE5 9RS

Tel: **020 3299 3601**

Email: **kch-tr.palsdh@nhs.net**

You can also contact us by using our online form at

www.kch.nhs.uk/contact/pals

If you would like the information in this leaflet in a different language or format, please contact PALS on 020 3299 1844.



