



Hydroxycarbamide (hydroxyurea) for teenagers

A patient's guide

As part of your treatment for your sickle cell disease, you have been prescribed a drug called hydroxyurea. It is very important that you are closely monitored while on the medication.

This is to make sure that you are on the correct dose. It is also very important that you take your prescribed medication correctly, whether it is in tablet or suspension (liquid form), so that it works best for you.

What is hydroxyurea?

For decades, hydroxyurea (also known as hydroxycarbamide) has been regularly used in the treatment of different blood disorders. There is a growing body of research evidence for the use of hydroxyurea in both children and adults with SCD and thalassaemia. The national standards of care listed below recommend the use of hydroxyurea in a variety of situations:

- “Standards for the Clinical Care of Adults with Sickle Cell Disease in the UK 2018”, and
- “Sickle Cell Disease in Childhood – Standards and Guidelines for Clinical Care 2010”.

It works by increasing the production of foetal (baby) haemoglobin in your blood. The amount of foetal haemoglobin in your blood is normally low, but if it is increased by taking hydroxyurea it can reduce the sickling episodes that you may have by stopping the sickle haemoglobin from sticking together inside the red blood cell.

By taking this medication it is hoped that you will have fewer, less severe and less painful crises. This will mean that you will feel better in yourself and will not need to be in hospital for long periods of time. You'll be able to spend more time at school or with your friends, doing things that you like.

How does it work?

It appears to work in at least three ways, but it is not clear which is the most important effect of taking the drug.

- It increases the production of foetal haemoglobin, as described above. This takes some weeks or months to take effect.
- It decreases the ‘stickiness’ of the young red cells therefore preventing the sickle cells from sticking to the blood vessel lining, which may start a crisis.



- It reduces the white blood cell count (neutrophil), which is often raised in people who have severe sickle cell disease. This may be important because the white cells produce chemicals, which can cause inflammation (swelling) and might speed up sickling. Having fewer of them makes this less likely.

How do I take it?

Hydroxyurea comes in a liquid (for children) or a capsule and is taken by mouth. Treatment begins on a low dose daily, usually at:

- 20mg/kg in a child
- 0.5g (one capsule) or 15mg/kg (rounded up to the nearest capsule) in an adult.

The dose will be gradually increased every two to three weeks until the right dose is achieved. This will be the dose that gives you the most benefits without side effects. The decision will be based on your symptoms and the blood test results. This process may take several weeks to complete, after which you will need blood tests every three months.

If there are any unwanted effects for you/, or in the blood tests, the hydroxyurea may be stopped or recommenced at a lower dose.

Patients who are sexually active must use contraception while taking hydroxyurea as its effects have not been formally tested while pregnant. They will also have to stop it for three months before trying to get pregnant. Once the woman is pregnant, the man can restart his hydroxyurea; however, if it is the woman who is taking it she will need to stay off hydroxyurea until the baby has been born and she has finished breastfeeding.

Patients unwilling to be monitored regularly will not be eligible to use hydroxyurea.

Giving my consent (permission)

If you agree to take hydroxyurea, or if you agree for your baby to be started on it, you will need to sign a consent form. If you are under 18 year of age, a parent or guardian may be required to sign the consent form, though you can sign it too.

What to expect

Most patients remain well, and have very little or no side effects. Hydroxyurea affects blood cells being produced so noticing any difference may take a few weeks. It is important that you do not give up taking hydroxyurea – it may take a couple of months before you reach the right treatment dose and can benefit from the effects of the medication.

Hydroxyurea occasionally causes sickness and vomiting, skin rashes, hair loss, diarrhoea, liver damage, weight gain, and if the blood count drops significantly, infection or bleeding. It may also cause darkening of the nails.

Many people ask if hydroxyurea increases the risk of cancer. We all have a risk of developing cancer during our lifetime. Some medications increase this risk a little, and this is true of a number of medications which work in a similar way to hydroxyurea. However, there is no evidence that the risk of cancer is increased in patients with sickle cell disease who take hydroxyurea.



Some groups of medications, such as hydroxyurea, should be avoided when planning to start a family. Therefore, adolescents and adults should discuss fertility, sperm bank collection and birth control on an individual basis with their consultant. There is no evidence that hydroxyurea affects fertility. As it has not been tested in large trials on pregnant women, we advise patients to stop it.

Risks

If you have pain, even severe pain, then you must continue to take the hydroxyurea. There are a number of side effects; hydroxyurea can lower the production of white blood cells and your platelet count. If you get a temperature over 38°C you should stop taking your hydroxyurea and arrange to have a blood test at the hospital urgently.

Increased incidence of leukaemia with hydroxyurea treatment is also a concern. This possible risk has to be weighed against the risk of severe sickle cell disease and the decision is often a difficult one.

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Date published: 07/02/2019

Review date: 07/02/2021

Ref: CYP/Paed/HT/02

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