Hydroxyurea (also known as hydroxycarbamide)

A patient's guide

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- Traditionally, the treatment for sickle cell disorder (SCD) was limited to pain relief and fluid for pain episodes, antibiotics for infection, and blood transfusions for serious complications.
- It was not possible to alter the course of the condition, except by bone marrow transplant.
- However, for the past 30 years, a medication called hydroxyurea has been found to help many patients.

What is hydroxyurea?

- For decades, hydroxyurea has been regularly used in the treatment of different blood disorders. Recently it has been used for SCD and thalassaemia.
- There is a growing body of research evidence for the use of hydroxycarbamide in children and adults and we now recommend that it is offered to all patients with Hb SS or Hb SBeta-zero, even those with no complications.
- It is also appropriate for other people with sickle cell disorders in other situations.

What does hydroxyurea do for patients with SCD?

- The best evidence we have comes from two large multicentre studies conducted in America.
- In the first study (known as the MSH trial and published in 1994), a large group of adult patients received either hydroxyurea capsules or an identical looking dummy drug (placebo).
- In the second study (known as the BABY HUG trial and published in 2012), either a placebo or hydroxyurea was given to infants with sickle cell disease.
- In both studies, neither the patients/parents nor the doctors knew which drug they were taking, so that the benefits and any side effects could be monitored objectively.
- Following both studies, significant improvement in health was noted in most patients receiving hydroxyurea compared to those taking the dummy drugs.
- The experiences were:
  - A longer pain-free period after starting treatment
  - Even longer period before the next painful episode
  - Fewer episodes of chest crises/sickle lung
  - Fewer blood transfusions
- Overall, the treated patients had fewer and less severe crises. About 8 out of 10 patients improved substantially. Unfortunately, it is not possible to predict which patients are likely to benefit most. More recently the BABY HUG study showed beneficial effects in babies treated from birth.

How does hydroxyurea work?

It appears to work in at least three ways:

- It increases the production of foetal (baby) haemoglobin, which is normally at a low level in adults, but if it is increased, it can reduce the frequency and severity of sickle cell crises. This takes some months or weeks to be effective.
- It decreases the stickiness of the young red blood cells. When they stick to the blood vessel lining, they may start a sickle cell crisis. This is thought to be the reason why some people notice that they feel better quite soon after starting treatment.
- It decreases the number of white blood cells and platelets, which are often raised in people with SCD. White blood cells produce a substance that causes inflammation and may worsen a sickle cell crisis. Having fewer white cells makes this less likely. Platelets also contribute to stickiness.

How do I take it and what monitoring and precautions are needed?

- Hydroxyurea comes in a liquid (for children) or a capsule and is taken by mouth. Treatment will begin on a low dose daily, usually 20mg/kg in a child or 500mg (one 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 results of your blood tests. This process may take several weeks to complete, after which you will need blood tests every one to two months.
- If there are any unwanted effects for you or in your blood tests, the hydroxyurea may be stopped or re-started 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 taking hydroxyurea 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, you will need to sign a consent form.
- If you are under 18 years 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 few 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 your medication – 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 several 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 group 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 before conception.

Contact details

- Your consultant team is:

  Dr Emma Drasar
  Dr Ryan Mullally
  Dr Ana Ortuno-Cabrero
  Dr Ali Rismani
  Dr Zara Sayar
  Dr Annabel McMillan
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- Adult departmental contacts:
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- Network website:
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If you or your family have any other questions, please do not hesitate to contact any of the above medical team at Whittington Health.

Patient advice and liaison service (PALS)

If you have a compliment, complaint or concern please contact our PALS team on 020 7288 5551 or whh-tr.PALS@nhs.net

If you need a large print, audio or translated copy of this leaflet please email whh-tr.patient-information@nhs.net. We will try our best to meet your needs.

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