If you or your family have any other questions please do not hesitate to contact any of the above medical team at University College London Hospital or The Whittington Hospital.

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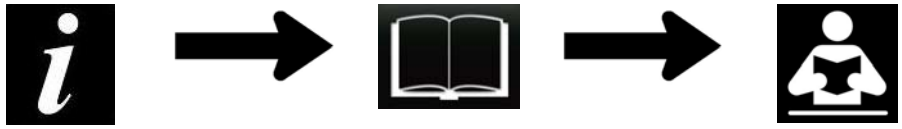
The Whittington Hospital NHS
NHS Trust

Patient information for Hydroxycarbamide

also known as Hydroxyurea

A patient's guide



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Until quite recently the treatment for sickle cell problems has been limited to pain relief and fluid for pain episodes, antibiotics for infection, and blood transfusions for serious complications. Nor has it been possible, until now to alter the course of the condition, except by bone marrow transplant. In the last few years a medication called Hydroxyurea has been found to help a significant proportion of patients.

Hydroxyurea can increase the production of fetal (baby) haemoglobin which is normally in adults at a low level but if it is increased, it can reduce sickling. We know this because at birth, when most of the blood is of the fetal type, sickling does not occur. Also, some people with sickle cell disease go on making higher than usual levels of fetal haemoglobin as adults and these people have fewer problems and live longer.

This medication is called Hydroxyurea, and most patients who have taken it have found that it definitely helps.

What is Hydroxyurea?
It is a drug which has been in regular use for decades, in the treatment of different blood disorders, but only recently, in the USA has it been used for sickle cell disease and thalassaemia. Although there is not yet a licence for use in these conditions, there is a growing body of research evidence for the use of Hydroxyurea in children and adults.

What does Hydroxyurea do for patients with SCD?
The best evidence we have comes from a large multi-centre study conducted in America, and published in 1994. A larger group of patients were entered into the study and received either Hydroxyurea capsules or an identical looking dummy drug (placebo).

Neither the patients nor the doctors knew which they were taking, so that the benefits and any side effects could be monitored objectively. All patients were carefully followed up for a period of nearly two years.

A majority of the patients receiving Hydroxyurea noted significant improvement in their health, as compared with those taking the dummy drugs.

The experiences were:

- A longer pain free period after starting treatment
- Even longer period before next painful episode
- Less episodes of chest crises/sickle lung
- The need for blood transfusion was less
- Overall the treated patients had fewer, less sever crises. About 80 percent did improve substantially. Unfortunately it is not possible to predict which patients are likely to benefit most.

How does Hydroxyurea work?

It appears to work in at least three ways:-

- It increases the production of fetal haemoglobin.
- 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. Hydroxyurea produces this effect much more quickly and this is though 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, which are often raised in people with sickle cell disease.
- White blood cells produce a substance which causes inflammation and may aggravate a sickle cell crisis. Having fewer white cells makes this less likely.

How do you take it?

Hydroxyurea comes in a capsule and is taken by mouth. Treatment will begin on a low dose daily 0.5 to l gram (1 to 2 tablets) increasing weekly using a treatment regimen/protocol (depending on your weight). This process may take several weeks to complete.

What to expect

Most patients remain well, but it is important that you are aware of possible side- effects from the drug. Hydroxyurea occasionally causes sickness and vomiting, skin rashes, hair loss, diarrhoea, liver impairment, weight gain, and if the blood count drops significantly, infection or bleeding. It may also cause darkening of the nails.

We all have a risk of developing some sort of malignancy during our lifetime. Some drugs increase this risk a little, and this is true of a number of drugs, which work in a similar way to Hydroxyurea. There is no evidence that this is the case in patients with sickle cell disease. However, it is something you should be aware of, and it may be something you will wish to discuss further with your consultant.

Some group of drugs 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.

Patients must use contraception whilst taking Hydroxyureal

Monitoring
To avoid the side effect of Hydroxyurea affecting your blood counts you will require blood test monitoring. This initially may be one - two weekly until maximum dosage of the drug is reached, then four weekly.

The purpose of the visits is to monitor and assess the effects of the drug, using blood tests and examination by the doctor. The drug will be stopped as soon as any unwanted effect on you or your blood is identified. The medication may be recommenced at a lower dose as soon as the blood counts are normal.

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

Consent
If you agree to take Hydroxyurea, you will be required to sign a consent form. If under 16 year of age, a parent or guardian will be required to sign the consent form.

Treatment with Hydroxyurea has been explained to you by Red Cell Disorders Unit.

Your Consultants are:

- Professor John Porter
- Dr Farrukh Shah
- Dr Bernard Davis
- Red Cell specialist registrar doctors
- Haemoglobinopathy consultant nurse specialist