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SCOTTISH PAEDIATRIC AND ADULT HAEMOGLOBINOPATHIES NETWORK

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Hydroxycarbamide (hydroxurea) in Sickle Cell Disease for the Parent/ Carer

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What is hydroxycarbamide?

Hydroxycarbamide is a drug which we use to treat a variety of blood and bone marrow diseases, including sickle cell disease. Hydroxycarbamide comes either as a capsule, a tablet or a liquid which your child will swallow.

How does hydroxycarbamide work in sickle cell disease?

Hydroxycarbamide has been used in large numbers of patients with sickle cell disease and has been shown to reduce the incidence of daily pain experienced at home or resulting in hospital admissions and life-threatening complications (chest crisis and stroke).

It can improve the quality of a patient's life by increasing exercise tolerance allowing more energy for daily activities.

There is now evidence that Hydroxycarbamide can increase long-term survival. It may also have a role in preventing long term lung and kidney damage.

It works by increasing fetal haemoglobin (the red cells that are seen in babies) and this protects against the damage caused by sickle cells. It also makes the blood less sticky by reducing the white cell count.

Hydroxycarbamide works for most patients but not all. We cannot predict who it will work for.

It may take up to 3 months for your child to get the full benefit of hydroxycarbamide.

Which sickle cell patients can be considered for hydroxycarbamide?

All children from the age of 9 months of age, with Sickle Cell Disease, who are Hb SS or Hb S/Beta thalassaemia, can be offered Hydroxycarbamide

- **To keep your child free from/reduce the complications of sickle cell disease**
- **To improve their life expectancy**
- **To improve their quality of life**

It is also especially important to offer your child Hydroxycarbamide if he or she:

- **has pain so frequent or bad that it stops them from having a normal quality of life**
- **has had several episodes of acute pain (usually three or more admissions to hospital or crises at home in a 12 month period)**
- **has had a life threatening crisis such as acute sickle chest syndrome**

How much hydroxycarbamide will my child take?

Your haematology doctor will decide your child's starting dose according to their weight. If your child tolerates it, your haematology doctor or nurse may increase the dose until he/she gets the full benefit.

It can be taken on an empty stomach or with food.

It should be given around the same time each day.

If you forget to give a dose, give the missed dose as soon as you remember. However, if it's nearly time for the next dose, skip the missed dose and then continue as usual.

Do not give a double dose to make up for a missed dose.

Do not give more than one dose in one day.

What side effects might my child experience?

With the improved well-being and survival, Hydroxycarbamide does have a few side effects

It may cause darkening of the skin and nails and rashes. These side effects are often mild.

Hydroxycarbamide can reduce the white count and platelet count to low levels and this may increase risk of infection and bleeding. As a result, the blood count needs to be monitored regularly until the dose is stable. This potential reduction in blood counts is reversible.

Contact your haematology doctor or nurse as soon as possible if you notice that

- **your child is more tired**
- **they have signs of infection such as a high temperature**
- **they have bruising or bleeding**

as your child will need to attend hospital for an assessment and blood tests.

There is no evidence that either sickle cell disease or hydroxycarbamide affect fertility. Hydroxycarbamide may reduce sperm production but evidence for this is unclear and no large studies have been done to prove this.

However, we do know that sperm production can differ in men with sickle cell disease, and this may be more likely after taking hydroxycarbamide. Boys should consider sperm testing and storage prior to taking hydroxycarbamide. Your Haematology doctor or Nurse may be able to arrange this for you.

Hydroxycarbamide may, theoretically, harm the unborn baby, although there is no clear evidence of this.

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Your doctor may advise your child to stop hydroxycarbamide when he/she is older and planning a pregnancy, but they may consider other treatments for them during this time.

The risks of becoming unwell after stopping hydroxycarbamide need to be weighed up with theoretical risks to the baby.

In the past there were concerns that hydroxycarbamide might increase the risk of leukaemia. However, a large study over several years did not show an increase in leukaemia in sickle cell patients who take hydroxycarbamide.

How will we monitor hydroxycarbamide?

Hydroxycarbamide can affect your child's blood levels of red cells, white cells, platelet count and liver tests. Therefore, we will monitor these blood levels. We will also monitor your child's HbF level.

When your child first starts their treatment, they will have blood tests regularly until they are on a stable dose. Once they are on a stable dose, we monitor their blood every 6 weeks to 3 months.

We will request a pregnancy test for girls who have started their period before starting hydroxycarbamide and at each clinic visit.

Prescriptions will not be supplied unless your child attends for these blood tests

Some frequently asked questions

Can hydroxycarbamide be prescribed in very young children?

Yes, hydroxycarbamide, can, and has been prescribed in very young children

My child cannot swallow tablets?

Hydroxycarbamide is available in liquid form upon request

Should I continue to give my child Hydroxycarbamide if he/she has a painful crisis?

Yes. You should continue to give Hydroxycarbamide as well as their usual medicine for pain.

My child has vomited after giving the Hydroxycarbamide. What should I do?

If you can see the whole hydroxycarbamide tablets, then repeat the dose once. If you cannot see the tablets or your child has taken liquid, do not repeat the dose. Call your doctor/nurse for advice.

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Can I get Hydroxycarbamide from my GP?

No you should not get Hydroxycarbamide from your GP. He/she will not be able to monitor your child's blood counts. Therefore, you should only get Hydroxycarbamide from your Haematology doctor/nurse.

Further Information

Scottish Paediatric and Adult Haemoglobinopathies Network (SPAH) through the website: spah.scot.nhs.uk

If you require an alternative format, please contact nss.equalitydiversity@nhs.scot, telephone: 0131 275 600

British Sign Language, please contact Scotland BSL: [Contact Scotland \(contactscotland-bsl.org\)](http://contactscotland-bsl.org)