

## 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 or a tablet. (If you are unable to swallow tablets, it can be given as a liquid).

## 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 you to get the full benefit of hydroxycarbamide.

## Which sickle cell patients can be considered for hydroxycarbamide?

Nearly all patients with Sickle Cell Disease, who are Hb SS or Hb S/Beta thalassaemia, can be offered Hydroxycarbamide

- To keep them 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 patients Hydroxycarbamide if they:

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

## How much hydroxycarbamide will I take?

Your haematology doctor will decide your starting dose according to your weight. If you tolerate it, your haematology doctor or nurse may increase the dose until you get 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 are the side effects of hydroxycarbamide?

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.

If you notice

- **That you are more tired**
- **signs of infection such as a high temperature**
- **bruising or bleeding**

contact your haematology doctor or nurse as soon as possible as you 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.

Your doctor may advise you (men and women) to stop hydroxycarbamide when planning a pregnancy but they may consider other treatments for you during this time. Therefore, it is important to discuss this with your doctor or nurse before stopping the medication.

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 blood levels of red cells, white cells, platelet count and liver tests. Therefore, we will monitor these blood levels. We will also monitor your HbF level.

When you first start the treatment, you will have blood tests regularly until you are on a stable dose. Once you are on a stable dose, we monitor their blood every 2 – 3 months.

**Prescriptions will not be supplied unless you attend for these blood tests**

## Some frequently asked questions

### I cannot swallow tablets?

Hydroxycarbamide is available in liquid form upon request

### Should I continue to take Hydroxycarbamide if I have a painful crisis?

Yes. You should continue to take Hydroxycarbamide as well as your usual medicine for pain.

### I have vomited after taking Hydroxycarbamide. What should I do?

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

### 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 blood counts. Therefore, you should only get Hydroxycarbamide from your Haematology doctor/nurse.



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## Hydroxycarbamide (hydroxurea) in Sickle Cell Disease for the young person

