



Factsheet

What is hydroxycarbamide?

Hydroxycarbamide is a drug that has been used for many years to treat blood disorders and certain types of cancer. It has also been found to be beneficial in SCD.

Findings from a research study conducted in the USA in the 1990s first showed that many sickle cell patients taking hydroxycarbamide have fewer painful crises, chest crises and blood transfusions. More recent evidence shows that hydroxycarbamide can also improve life expectancy in SCD.

Hydroxycarbamide is usually recommended if you have had three or more hospital admissions in the past year for a sickle cell crisis, have regular crises at home affecting work or normal daily life or have had two or more chest crises.

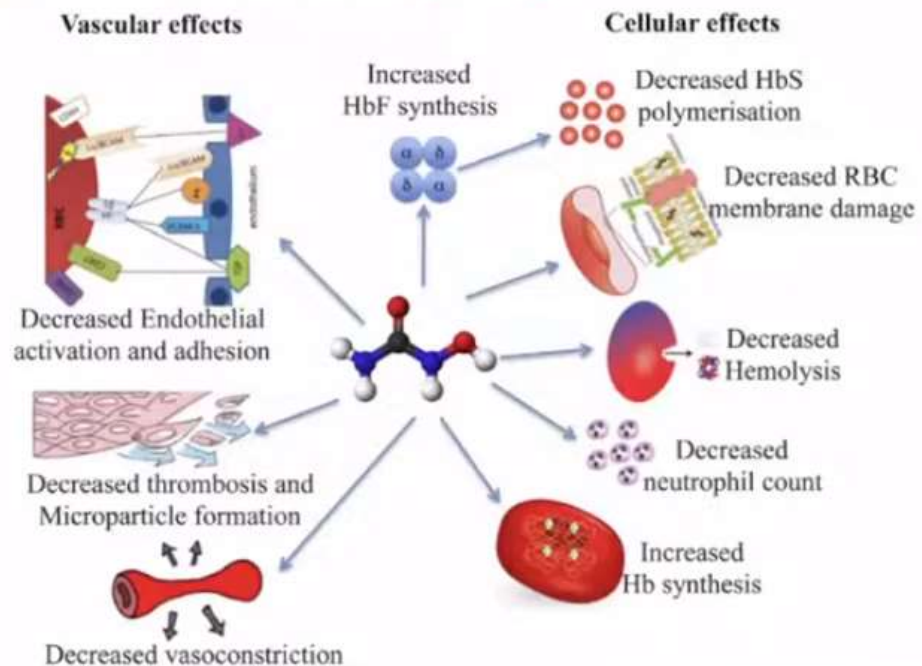
It is currently the only effective anti-sickling drug but underused in many countries

How does hydroxycarbamide work in SCD?

Hydroxycarbamide works in several ways by:

1. Increasing the number of red blood cells containing fetal haemoglobin (HbF). Having more HbF is a good thing in SCD. During the first few months of life red blood cells contain mostly HbF. This protects against sickling. As the level of HbF falls the benefit wears off. Some people with SCD naturally produce more HbF throughout life. They have fewer problems and live longer. Hydroxycarbamide stimulates production of HbF which reduces sickling – this effect may take a few months.
2. Helping to keep red cells well-hydrated and reducing their stickiness. This may prevent a crisis developing. These effects occur quite quickly which may explain why some people feel better and experience less pain within a few weeks of starting treatment.
3. Reducing the number of white blood cells which is often high in people severely affected by SCD. These cells produce chemicals that can cause inflammation and trigger a crisis. Having fewer white cells makes sickling less likely.

Hydroxyurea in sickle cell disease (SCD)



Are there any side effects associated with hydroxycarbamide?

Most people tolerate hydroxycarbamide treatment well but, like all medicines, there are some potential side effects. The risk of side effects is reduced by seeing you regularly in the clinic and checking your blood counts. Most side effects are reversible on stopping or reducing the medication.

Low blood counts are a side effect you should be aware of. If this happens you may notice you:

- are more tired or short of breath due to a fall in haemoglobin.
- bruise or bleed more easily due to low platelets (a type of blood cell important in blood clotting)
- develop mouth ulcers or pick up infections more easily due to low white blood cells.

You may not initially be aware that your blood counts are low so it is important to attend your clinic appointments for these to be checked. You can discuss any other symptoms that you are concerned about with your doctor at these appointments.

Other unwanted effects that can occur include an upset stomach (diarrhoea, constipation or sickness), poor appetite, headaches or dizziness, allergic reaction such as skin rash, skin and nail pigmentation, hair thinning, skin ulceration, abnormal liver or kidney function tests. Most of



these are uncommon.

There have been concerns that hydroxycarbamide may increase the risk of developing leukaemia and other cancers. However, after more than 20 years' experience of its use in SCD internationally, there is no evidence to support this.

If you are taking hydroxycarbamide you or your partner should use condoms even if another form of contraception is used. This protects you or your partner from exposure to the small amounts of the drug present in semen or vaginal secretions and any possible harm that might result.

The World Health Organization (WHO) estimates that more than 70% of SCD related deaths are preventable with early diagnosis and widely available cost-efficient interventions (e.g., pneumococcal vaccinations, daily penicillin, hydroxyurea). For example, hydroxyurea use has been shown to reduce the vaso-occlusive crises, stroke, infections, malaria, transfusions, and death in children with SCD, warranting the need for wider access to this treatment. Hydroxyurea treatment has been shown to be feasible, safe, and effective in children with SCD living in India and in sub-Saharan Africa.



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