



**SPAHA**

SCOTTISH PAEDIATRIC AND ADULT HAEMOGLOBINOPATHIES NETWORK

# Information for patients, relatives and carers

## Hydroxycarbamide treatment in adults with sickle cell disease

This document has been prepared by NHS National Services Scotland (NSS) on behalf of SPAHA. Accountable to Scottish Government, NSS works at the heart of the health service providing national strategic services to the rest of NHS Scotland and other public sector organisations to help them deliver their services more efficiently and effectively. The SPAHA Network is a collaboration of stakeholders involved in care of patients with haemoglobinopathies, who are supported by an NSS Programme Team to drive improvement across the care pathway.

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## Introduction

This information has been provided to answer some of the questions you may have about treatment with hydroxycarbamide (also known as hydroxyurea) in sickle cell disease (SCD).

Together with advice from your specialist team this will help you to make an informed decision about taking hydroxycarbamide.

## What is hydroxycarbamide?

Hydroxycarbamide is a medicine used for many years to treat blood disorders. 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 experienced fewer pain 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
- you have had two or more episodes of acute chest crises.

There may be other reasons why hydroxycarbamide could benefit you and this will be discussed with you by your haematology team.

## How hydroxycarbamide works in SCD?

Hydroxycarbamide works in three main ways.

1. Increasing the amount of fetal haemoglobin (HbF) in red blood cells which makes it harder to form the sickle shape.
  - 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 level of haemoglobin S (HbS – sickle haemoglobin) rises and red cells are increasingly susceptible to sickling. Some people with SCD naturally produce more HbF throughout life. They have fewer problems and longer life expectancy.
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.

It may take 3 to 6 months to feel the full benefits and so it is important to give it time to work.

## How to take hydroxycarbamide

- Hydroxycarbamide usually comes in 500 mg capsules or tablets and is taken by mouth once a day.
- After starting treatment, the dose of hydroxycarbamide is usually increased gradually. This will be monitored and adjusted when you attend the outpatient clinic. The maximum dose depends on your weight, blood counts and how you respond to the drug. For adults, the maximum dose is usually three to four capsules per day. Increasing to the maximum dose that works for you allows the greatest benefit to be achieved.
- Take it at the same time each day, with or without food.
- Swallow capsules whole with water.
- Take care not to break open the capsule. If this happens accidentally, wash your hands straight away.
- If you struggle to swallow capsules, ask your clinical team about other options.
- It is important to take hydroxycarbamide as prescribed to ensure you get the most benefit from treatment and avoid unwanted effects.
- If you miss a dose, take the next dose as usual and don't double it.
- If you have been unable to take hydroxycarbamide regularly, it is important that you tell your clinical team at your next clinic appointment.
- Please note, that unlike most medications, hydroxycarbamide will usually be prescribed by the hospital team rather than your GP. If you think you may run out of capsules before your next clinic appointment, contact your clinical team. They will be able to arrange a prescription.
- If you take too much by mistake, call your haematology team urgently.

## How to store and dispose of hydroxycarbamide

- Keep it in a dry place at room temperature.
- Make sure it is placed in a secure location, out of the reach of children and pets. Like many drugs, hydroxycarbamide is harmful and can be dangerous if taken by people or pets when not prescribed.
- Return any unused medication to your hospital or local pharmacy.
- Don't throw the capsules in the bin or flush them down the toilet.

## Side effects

Most people do well on hydroxycarbamide, but like all medicines side effects can happen. 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.

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Watch out for signs of low blood counts. This is the reason we check your blood counts initially and aim to prevent this. If this happens you may note 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.

If you experience any of the symptoms listed above or develop a fever greater than 38°C (100.4°F) you should contact the haematology department or go to the nearest A&E.

You may not be aware if your blood counts are low to start with, 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 or CNS at these appointments.

Other unwanted effects are either very mild or rare and 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.

**Please remember that there are potential side effects with any medication which can sound worrying. It is, however, important to balance these with the benefits that hydroxycarbamide offers.**

## Taking other medicines

It is safe to take hydroxycarbamide with most other medications. You should let your doctor or CNS know about all other medications you are taking or are prescribed.

If you're planning to get a live vaccine (like yellow fever or shingles), speak to your haematology doctor first.

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### Food and drink

There is no reason to change your diet when taking hydroxycarbamide. Alcohol is allowed but stick to the recommended limit of 14 units of alcohol per week.

### If you or your partner would like to would like to become pregnant?

Hydroxycarbamide treatment doesn't stop you or your partner from having children. But if you're planning a pregnancy, talk to your doctor first. Hydroxycarbamide should be stopped for three months before conception to avoid any potential risk to the baby. Use reliable contraception while on treatment.

If you or your partner becomes pregnant while taking hydroxycarbamide contact your doctor or CNS as soon as possible for further advice.

There is no evidence that taking hydroxycarbamide reduces fertility although in men the sperm count may be reduced while taking the drug. This usually reverses on stopping treatment. As a precaution we advise you to consider sperm analysis and storage before starting treatment.

### Breastfeeding while taking hydroxycarbamide

You should not breastfeed while taking hydroxycarbamide. The medicine can pass into breast milk and may affect your baby.

### Other precautions

Use sunscreen: your skin may be more sensitive to sunlight while on hydroxycarbamide, so protect it with sunscreen.

Use condoms: even if you're using another form of contraception, condoms help protect your partner from small amounts of the drug that may be present in semen or vaginal fluids.

### Consent

Before starting hydroxycarbamide, your doctor or CNS will explain the benefits and risks. You may be asked to sign a consent form to confirm you understand and agree to the treatment.

For more information about SPAH: SPAH Website: [www.spah.scot.nhs.uk](http://www.spah.scot.nhs.uk)

Email: [nss.spah@nhs.scot](mailto:nss.spah@nhs.scot)

If you need this leaflet in another format, please contact [NSS.EqualityDiversity@nhs.scot](mailto:NSS.EqualityDiversity@nhs.scot), 0131 275 6000

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Thank you to the West London Haemoglobinopathy Coordinating Centre for allowing us to use and adapt content from their original materials in this leaflet.