NHS SCOTLAND

SPAH
SCOTTISH PAEDIATRIC AND ADULT HAEMOGLOBINOPATHIES NETWORK

Hydroxycarbamide treatment in children with sickle cell disease

Information for parents and carers

This document has been prepared by Public Services Delivery Scotland (PSD Scotland) on behalf of the Scottish Paediatric and Adult Haemoglobinopathies Network (SPAH). Accountable to Scottish Government, PSD Scotland 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 SPAH Network is a collaboration of stakeholders involved in care of patients with haemoglobinopathies, who are supported by a PSD Scotland Programme Team to drive improvement across the care pathway.

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Introduction

This leaflet 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 child's specialist team, it will help you make an informed decision about your child starting 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 patients with SCD taking hydroxycarbamide experienced fewer pain crises, chest crises and blood transfusions. More recent evidence shows that hydroxycarbamide can also improve life expectancy.

Who can be considered for hydroxycarbamide?

Children aged nine months and older with SCD (types HbSS or HbS/β) should be offered hydroxycarbamide to:

- help prevent or reduce complications of SCD and organ damage
- improve life expectancy
- help them feel better and enjoy a better quality of life

Doctors should recommend hydroxycarbamide if your child:

- experiences frequent or severe pain that affects their quality of life
- has been admitted to hospital three or more times in the past year due to sickle cell crisis
- has regular crises at home that disrupts school or daily routine
- has a life-threatening crisis such as acute chest syndrome

There may be other reasons why hydroxycarbamide could benefit your child and these will be discussed with your child's 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 cause inflammation and trigger a crisis. Having fewer white cells makes sickling less likely and may also protect organ damage.

Hydroxycarbamide works for most children though not all. We cannot predict who it will work for. 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?

- The starting dose is based on your child's weight.
- If your child is doing well on it, the doctor or nurse will increase the dose to make sure they get the best possible benefit. This will be monitored and adjusted during clinic visits.
- Take it at the same time each day, with or without food.
- Capsules should be swallowed whole with water.
- Take care not to break open the capsule. If this happens accidentally, wash your hands straight away.
- If your child struggles to swallow capsules, ask their clinical team about other options.
- It is important your child takes hydroxycarbamide as prescribed to ensure they get the most benefit from treatment and avoid unwanted effects.
- If a dose is missed, give it as soon as remembered unless it's close to the next dose – then skip it. Never give two doses in one day.

- Inform the haematology team if doses are missed or if you're running low on medication.
- Inform the haematology team if your child has been unable to take hydroxycarbamide regularly.
- Contact the haematology team immediately if you give too much hydroxycarbamide by mistake.

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 children do well on hydroxycarbamide, but like all medicines side effects can happen. The risk of side effects is reduced by the haematology team seeing your child regularly in the clinic and checking their blood counts. Most side effects are reversible on stopping or reducing the medication.

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

- is more tired or short of breath due to a fall in haemoglobin
- bruises or bleeds more easily due to low platelets (a type of blood cell important in blood clotting)
- develops mouth ulcers or picks up infections more easily due to low white blood cells

If your child experiences any of the symptoms listed above or develops a fever greater than 38ºC (100.4ºF) you should contact the haematology department or go to the nearest A&E immediately.

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

Other unwanted side effects are either very mild or rare and include:

- upset stomach (diarrhoea, constipation or sickness)
- poor appetite
- headaches or dizziness
- allergic reaction such as skin rash
- skin or nail pigmentation
- hair thinning
- skin ulcers
- 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 child's doctor or CNS know about all other medications they are taking or are prescribed.

Speak to the haematology doctor prior to your child being given a live vaccine (like yellow fever or shingles).

Food and drink

There is no reason to change your diet when taking hydroxycarbamide.

Fertility

There is no evidence that hydroxycarbamide affects fertility. In the future if children are planned, they should discuss this first with their haematologist or CNS.

Hydroxycarbamide should be stopped for at least three months before conception to avoid any risk to the baby.

In boys, sperm count may be reduced while taking the drug. This usually reverses on stopping treatment. As a precaution we advise boys, who are old enough to do so, to consider sperm analysis and storage before starting treatment.

Other precautions

Sunscreen: remember to use sunscreen to protect their skin. It may become more sensitive to the sun while they are taking hydroxycarbamide.

How will we monitor hydroxycarbamide?

Hydroxycarbamide can affect your child's blood, so we'll monitor it closely. It may change levels of red and white blood cells, platelets, and liver function. That's why we'll carry out regular blood tests to check these. We'll also monitor your child's fetal haemoglobin (HbF) level, which helps protect again SCD complications.
When your child first starts treatment, blood tests will be done frequently – until we find the dose that works well and is safe. Once stable, blood tests will be done every 6 weeks to 3 months.

For girls who have started their periods, a pregnancy test will be done before starting hydroxycarbamide and at each clinic visit.

Prescriptions will only be supplied if your child attends these blood test appointments.

Some frequently asked questions

Can hydroxycarbamide be given to very young children?

Yes. It is safe and has been used effectively in very young children with SCD.

What if my child can't swallow tablets?

Hydroxycarbamide is available as a liquid. Just ask your healthcare team.

Should I give hydroxycarbamide during a painful crisis?

Yes. Continue giving hydroxycarbamide as usual, along with any pain relief your child normally takes.

What if my child vomits after taking hydroxycarbamide?

If you can see the whole tablet in the vomit, you may give the dose again. If you can't see the tablet or your child took the liquid form, don't give another dose. Call your doctor or nurse for advice.

Can I get hydroxycarbamide from my GP?

No. Your GP can't monitor the blood test required for this medicine. You should only get hydroxycarbamide from your child's haematology doctor or nurse.

For more information about SPAH and other helpful resources:

SPAH Website: www.spah.scot.nhs.uk
Email: nss.spah@nhs.scot

If you need this leaflet in another format (such as large print), please:

- Email: psd.contact-us@nhs.scot
- Phone: 0131 275 6000
- Contact: BSL ContactScotlandBSL at contactscotland-bsl.org.

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

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