Hydroxycarbamide for the treatment of sickle cell disease

Information and advice for patients

Sickle Cell & Thalassaemia Centre, Haematology

Until recently, the treatment of sickle cell disease (SCD) has been limited to giving pain relief and fluids for managing pain as well as antibiotics for infection and blood transfusions for serious complications. Hydroxycarbamide has been in use for a few years and has been shown in some cases to improve the symptoms of SCD.

Please note hydroxycarbamide is sometimes also known as hydroxyurea.

What is hydroxycarbamide?

Hydroxycarbamide is used in the UK largely for patients with repeated painful crises, those with 2 or more chest crises (lung sickling). It has been shown to be effective for treating symptoms of SCD. It is given in the form of capsules which are taken by mouth.

How does it work?

Hydroxycarbamide has a number of effects on sickle cell , these include:

- Increasing the amount of foetal (baby) haemoglobin (HbF). This can be beneficial as some patients with SCD who have higher natural levels of HbF generally have milder symptoms.
- Increasing the amount of water in the sickle cell. This makes it more difficult for the red blood cell to develop a sickle shape.
- Reducing the ability of the red blood cell to stick to the lining of the blood vessel. This is beneficial as red blood cells stick to the lining of the blood vessel a crisis may start.
- Reducing the white blood cell (neutrophil) count, which is often higher in patients with SCD. This is important because the white blood cells produce chemicals that can cause inflammation and might trigger sickling; having fewer neutrophils makes this less likely.
- Increasing the levels of various substances (e.g. nitric oxide) in the blood which helps with blood flow through the tissues of the body.

How soon will it work?

You will need to be on it for at least 6 months before you can be sure of its benefits. Although most patients will respond to hydroxycarbamide, not all do and it is not possible to predict which patients are and are not likely to benefit.

What are the benefits of hydroxycarbamide?

Hydroxycarbamide can reduce the complications of SCD. It is used in adults and adolescents with moderate to severe repeated pain. Hydroxycarbamide can reduce the number and severity of painful crises, the number and severity of chest crises (lung sickling), the number of blood transfusions, and the number of admissions to hospital. Long-term follow up has shown that patients taking this treatment are in better physical health.

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What are the risks and side effects?

The risks associated with hydroxycarbamide are low, but there are some side effects to be aware of:

- If you take too high a dose, it can cause a fall in your haemoglobin level or white blood cells. You will start on a low dose and have regular blood tests during your treatment; it is important that you attend to have these checks.
  - Lowering the white blood cells can make you more likely to develop an infection. If you develop a fever (temperature above 38°C), sore throat, or if you suddenly feel unwell, you should stop the tablets and contact your doctor to have a blood test to check whether your blood count has fallen. This does not happen often; however, if it does you may need treatment with antibiotics. The blood count tends to go back to normal in a few days
  - Bruising or bleeding. Hydroxycarbamide reduces your platelets, which helps your blood to clot. If your platelets are too low, you can develop bruising and bleeding which you should report to your doctor.
- Other side effects are less common and include the following: nausea, diarrhoea, constipation, darkening of skin and nails, and temporary hair loss.
- There is a very low risk of developing leukaemia or cancer, but there has not been enough research to prove this.

What are the risks of not taking the medication?

By not taking the medication you may prevent seeing an improvement in your symptoms.

Are there any alternatives to this medication?

Whilst there are other alternatives to hydroxycarbamide, this is considered to be the safest option.

How to take the medication

It is important to read the leaflet that comes with the medication before taking it for the first time. You should take the correct dosage of the medication with water. If the capsule is opened or crushed, it is important to avoid touching this – you should wear gloves to clean this. Use only the dose prescribed and check the expiry date.

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Will I need any extra tests?

You will have a blood test at the start of treatment and every 2 weeks after this. Once you are stable on treatment, the blood tests will become less frequent.

Precautions

Women who are pregnant or breast-feeding should not take hydroxycarbamide as it can harm the baby. If you are taking hydroxycarbamide you should use contraception to ensure that you do not become pregnant. If you are on hydroxycarbamide (male or female) and you are planning a family, should inform your doctor and stop hydroxycarbamide 3 months before.

Storing the medication

You should store this medication at 25°C and keep out of reach of children.

Contact details

If you have any questions or concerns please contact the Sickle Cell and Thalassaemia Centre.

Sickle Cell & Thalassaemia Centre
Sandwell & West Birmingham Hospitals
City Hospital
Dudley Road
Birmingham
B18 7QH

Tel: 0121 507 6040

Monday, Wednesday & Thursday 9am – 5pm
Tuesday 9am – 6pm
Friday 9am – 4pm

It is advisable to compare the risks and benefits with any other treatments that you may be receiving to manage your condition. If you require further information, you can contact the Haematology team to discuss further the information in this leaflet and any other questions that you may have about hydroxycarbamide.

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For further information

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For more information about our hospitals and services please see our websites *www.swbh.nhs.uk* and *www.swbhengage.com*, follow us on Twitter *@SWBHnhs* and like us on Facebook *www.facebook.com/SWBHnhs*.

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Sources used for the information in this leaflet

- British National Formulary, '9.1.3 Drugs used in hypoplastic, haemolytic and renal anaemias – Sickle-cell Disease - Hydroxycarbamide', Accessed 12th May 2014
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- Ware R.E. and Aygun B., Hematology American Society of Hematology Education Program, 'Advances in the use of hydroxyurea', 2009.
- DailyMed, 'Hydroxyurea capsule [Par Pharmaceutical, Inc.]', Accessed 19th May 2014.

If you would like to suggest any amendments or improvements to this leaflet please contact the communications department on 0121 507 5495 or email: **swb-tr.swbh-gm-patient-information@nhs.net**

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