Hydroxycarbamide for the treatment of sickle cell disease

Information and advice for patients

Sickle Cell and Thalassaemia Centre, Haematology

Until recently, the treatment of sickle cell disease (SCD) has been limited to giving pain relief and fluids for dealing with pain, 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.

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.

What are the benefits of hydroxycarbamide?

Hydroxycarbamide can reduce the complications of SCD. Hydroxycarbamide is used in adults and adolescents with moderate to severe repeated pain. Hydroxycarbamide has been shown to 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.

How does it work?

Hydroxycarbamide has a number of effects on sickle cell:

1. It increases 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.
2. Hydroxycarbamide increases the amount of water in the sickle cell. This makes it more difficult for the red blood cell to develop a sickle shape.
3. Hydroxycarbamide reduces the ability of the red blood cell to stick to the lining of the blood vessel. This is beneficial as when red blood cells stick to the lining of a blood vessel a crisis may start.
4. Hydroxycarbamide reduces 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.

5. Hydroxycarbamide also increases 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 take hydroxycarbamide for at least 6 months before you can be sure of its benefits. Most patients will respond to hydroxycarbamide, but it does not work for everybody and it is not possible to predict which patients will benefit.

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 38oC), 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.
- Many people are concerned about the risk of developing leukaemia or cancer. We all have a risk of developing leukaemia or some other form of cancer in our lifetime. Hundreds of people around the world take hydroxycarbamide, and some people have been on this treatment for many years. If this risk exists with hydroxycarbamide, it is likely to be low.

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.

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 the 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 and Thalassaemia Centre
C3, 3rd Floor
Midland Metropolitan University Hospital (MMUH)
Grove Lane
Smethwick
B66 2QT

Telephone: 0121 507 6040

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

Saturday blood transfusion service only

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 or have any other questions about hydroxycarbamide you can contact the Sickle cell and Thalassaemia centre team.

For further information

Birmingham Sickle Cell & Thalassaemia Service

Nineveh House,
Nineveh Road,
Handsworth, Birmingham,
B21 0SY

Telephone: 0121 466 3667

bchc.sicklecellresults@nhs.net

NHS Website (2022). Sickle cell disease. Available at: **https://www.nhs.uk/conditions/sickle-cell-disease/** [Accessed 16 October 2023].

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Sources used for the information in this leaflet

- British National Formulary (July 2020) Anaemias. [Online] Available at: **https://bnf.nice.org.uk/treatment-summary/anaemias.html** [Accessed 16 October 2023].
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- Ware, R. E., & Aygun, B. (2009). 'Advances in the use of hydroxyurea'. *ASH Education Program Book*, 2009(1), 62-69.
- Sickle Cell Society (2018) Standards for the Clinical Care of Adults with Sickle Cell Disease in the UK. Available at: **https://www.sicklecellsociety.org/wp-content/uploads/2018/05/Standards-for-the-Clinical-Care-of-Adults-with-Sickle-Cell-in-the-UK-2018.pdf** [Accessed 16 October 2023].

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