Hydroxyurea treatment in sickle cell disease

A guide for parents and carers of children and young adults

This leaflet offers further information about hydroxyurea, which may be used to treat sickle cell disease. It explains what hydroxyurea is, how it can help and lists a number of risks and side effects. If you have any further questions or concerns, please contact us using the details given at the end of this leaflet.

What is hydroxyurea?

Hydroxyurea is a medicine useful in the treatment of sickle cell disease (SCD). Studies have shown that it reduces both the number of painful crises and the number of chest crises in patients with sickle cell anaemia (SCA, a variety of SCD). Hydroxyurea can be used for both adults and children with SCA (Hb SS or Hb S-beta-thalassaemia). It has been used over the past 10 to 15 years to treat people with SCA, and has been used for decades to treat other blood conditions.

Why should my child take hydroxyurea?

Hydroxyurea is effective in about 80% of people who take it. Benefits include:

- reduced number of painful crises
- reduced severity of pain
- reduced number of chest crises.

It is also possible that hydroxyurea can reduce damage to blood vessels and reduce instances of stroke.

How does hydroxyurea work?

Hydroxyurea works in a number of ways to reduce the sickling tendency of red blood cells.

- Hydroxyurea increases the level of foetal haemoglobin (HbF) in red blood cells. This reduces sickling and may allow patients to have fewer problems and live longer.
- Hydroxyurea decreases the white blood cell count. White cells are large and sticky and increase the tendency of sickle cells to block blood vessels. Reducing the number of white blood cells may also reduce the amount of sickling.
- Hydroxyurea increases haemoglobin and therefore increases the amount of oxygen the blood delivers to the tissues.

How is hydroxyurea taken?

Hydroxyurea is taken by mouth once daily. It comes in capsules, but can be given in a liquid form if absolutely necessary. It is started at a low dose and then gradually increased, depending on the blood test results and your body’s response to the medication. Some people will notice an improvement to their SCD on a small dose of medication, but others will need a higher dose to feel better.
Your blood will be tested regularly at first, but will need to be checked less frequently (once every eight to twelve weeks) once you have been on the right dose of medication for a couple of months.

It usually takes three to six months of taking the right dose to find out if the medication is useful and suitable for you. It is important that you take your medication daily and that you do not stop taking it without advice from your doctor.

**Are there any side effects?**

You may be advised to stop taking hydroxyurea if any unwanted side effects on the blood are noted. The medication can be restarted once the blood tests are normal.

Hydroxyurea reduces the white blood cell count and the platelet count in the blood. This is generally a good thing, however, if the white cells get too low there is an increased risk of infection and the hydroxyurea may need to be stopped temporarily. Once the white count has increased, the medication can be restarted.

Other, less common side effects include:

- a darkening of the skin and nails, which goes away when the hydroxyurea is stopped
- mild sickness and/or nausea
- diarrhoea
- mild hair loss.

**Are there any risks to taking hydroxyurea?**

Hydroxyurea is a form of mild chemotherapy which helps with SCD because of the effect it has on bone marrow. Everyone has a low risk of developing leukaemia or some form of cancer in their lifetime and some forms of chemotherapy may increase this risk. There is no evidence that hydroxyurea can cause leukaemia in patients with SCD or in other groups of patients who have taken the medication for up to 20 years, however, there is a theoretical risk of damage to the bone marrow.

Hydroxyurea should be avoided when planning to start a family.

**For young men**

Hydroxyurea may affect the quality of sperm produced and this may cause a problem for the baby if you get someone pregnant while taking it. It is important to use contraception while taking hydroxyurea and to stop taking the medication three months before planning to start a family.

**For young women**

It is important not to get pregnant while taking hydroxyurea. It should also not be taken for three months before planning to get pregnant. It is important to use contraception while taking hydroxyurea if you are sexually active.

**Are they any alternatives?**

Bone marrow transplantation is the only cure for SCD, but for many children, hydroxyurea is the best option for helping to treat SCD. Whether or not your child takes hydroxyurea is your/your child’s decision – it will only be prescribed following discussion between you and your child’s consultant.
Useful contacts

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<thead>
<tr>
<th>Department</th>
<th>Telephone number</th>
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<tbody>
<tr>
<td>Childrens outpatient department (Dragon)</td>
<td>020 8725 5292</td>
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<tr>
<td>Haemoglobinopathy counsellor</td>
<td>020 8812 0636</td>
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<tr>
<td>Paediatric haematology secretary</td>
<td>020 8725 3921</td>
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If you wish to contact the paediatric haematology consultant, please contact the paediatric haematology secretary.

Additional services

Patient Advice and Liaison Service (PALS)
PALS can offer you on-the-spot advice and information when you have comments or concerns about our services or the care you have received. You can visit the PALS office between 9am and 5pm, Monday to Friday in the main corridor between Grosvenor and Lanesborough Wing (near the lift foyer). **Tel:** 020 8725 2453  **Email:** pals@stgeorges.nhs.uk

NHS Choices
NHS Choices provides online information and guidance on all aspects of health and healthcare, to help you make decisions about your health.
**Web:** www.nhs.uk

NHS 111
You can call 111 when you need medical help fast but it’s not a 999 emergency. NHS 111 is available 24 hours a day, 365 days a year. Calls are free from landlines and mobile phones.
**Tel:** 111