

# Hydroxycarbamide for children with sickle cell disease

## Information for families

### What is hydroxycarbamide?

Hydroxycarbamide is a medication which has been found to reduce the severity of painful crises in those with sickle cell disease (SCD). It is taken by mouth regularly once daily, either in capsule form (hydroxyurea), as a liquid, or as a tablet (siklos).

### How is it used?

Hydroxycarbamide is a mild cancer treatment which has been found to be beneficial in those with SCD. It has been used for the treatment of SCD for over 25 years. Treatment is started using a low dose, and then increased slowly until the most effective dose is achieved.

### How does it work?

Hydroxycarbamide has been found to raise the levels of foetal (or 'baby') haemoglobin F (HbF) in red blood cells. This type of haemoglobin (Hb) holds the oxygen better than other haemoglobins.

### How does it help SCD?

Because the HbF takes longer to release the oxygen, it reduces the tendency for sickle cells to clump together. This means that there will be less 'sickling', and less frequent or severe crises. Hydroxycarbamide also lowers the number of white blood cells (WBC), platelets and other blood cell types, which are also thought to have a part to play in sickle cell crises.

### Why should my child take hydroxycarbamide?

The doctor will offer hydroxycarbamide therapy to your child if they are considered to present with severe problems. That is, if they have had more than three severe crises in the past year, acute chest syndrome, and/or repeated infections. However, we will support the decision you make about whether or not you want to give hydroxycarbamide to your child.

### What difference will it make to my child?

You will not notice a difference immediately. Generally, it takes a couple of months for the HbF to build up. If you continue to give your child the medication regularly every day, you will notice that your child looks less pale and begins to have more energy. As the HbF level increases your child will have less severe painful crises.

## What dose will be used?

A small dose of 20mg/kg is given to start with. Your child will be monitored frequently with blood tests. The levels of HbF and WBC will tell us if the medication is working. The consultant will then increase the dose until it reaches the required amount. Thereafter, your child will need a blood test every two months while they are on the medication.

## What are the side effects of hydroxycarbamide?

Hydroxycarbamide can suppress the bone marrow and therefore reduce the number of circulating WBC and platelets. If these go too low, the risk of infection can increase. These are being monitored regularly with blood tests every two months and the medication dosage may be changed if necessary. Regular clinic follow-up is therefore very important.

Other side effects are not common, but if your child experiences increased tiredness, bleeding or bruising, or if they have a high temperature, you should seek medical advice or contact the sickle cell team.

Minor side effects like nausea and vomiting, sore mouth, thinning hair, rash or diarrhoea are usually mild, short-term and also uncommon. The manufacturer's patient information leaflet that comes with the medicine will contain further information on side effects.

## Long-term side effects

There are some concerns that hydroxycarbamide may increase the risk of secondary cancer and infertility. However, at present we are not aware of any research that proves these concerns are justified. Also, there is some research to show that children who take hydroxycarbamide are no more likely than any other child to develop leukaemia.

Research shows that hydroxycarbamide is safe in children from the age of nine months. It shows that the benefits of taking the medicine include:

- reduction in pain
- fewer blood transfusions
- fewer hospital admissions
- better overall health.

We will continue to monitor patients closely and will tell you about any relevant research findings when you see the consultant in your regular appointment. If you have any concerns about the possible long-term side effects of this treatment, please discuss them with the sickle cell team.

## What should I do now?

If you are interested in giving your child hydroxycarbamide, speak to your consultant or you can discuss it with another member of the sickle cell team.

## Taking an unlicensed medicine

Siklos® (hydroxycarbamide tablets) is the only hydroxycarbamide product that is licensed in the UK for the treatment of SCD. Other hydroxycarbamide products are available in the UK for the treatment of other conditions and although they are not licensed for SCD they may be more appropriate for your child. For more information on unlicensed medicines, please ask your doctor, nurse or pharmacist for a copy of our leaflet **Unlicensed medicines – a guide for patients**. Alternatively, you can call the Evelina London Medicines Helpline – contact details are at the end of this leaflet.

## Contact us

If you have any questions or concerns about hydroxycarbamide treatment, please contact the sickle cell team (Monday to Friday, 9am to 5pm):

- Consultant haematologist **t** : 020 7188 7774
- Adolescent sickle cell clinical nurse specialist (CNS). Call the hospital switchboard on 020 7188 7188 and ask for the bleep desk. Ask for bleep 2256 and you will be connected directly.
- Paediatric Haematology CNS **t**: 020 7188 4486 or call the hospital switchboard and ask for the bleep desk. Ask for bleep 2733 and you will be connected directly.
- Deputy Paediatric Haematology CNS **t**: 020 7188 9432
- Out of hours, please call the hospital switchboard on 020 7188 7188 and ask them to bleep the paediatric registrar.

For more information leaflets on conditions, procedures, treatments and services offered at our hospitals, please visit [www.evelinalondon.nhs.uk/leaflets](http://www.evelinalondon.nhs.uk/leaflets)

### Evelina London Medicines Helpline

If you have any questions or concerns about your child's medicines, please speak to the staff caring for them or contact our helpline.

**t**: 020 7188 3003 10am to 5pm, Monday to Friday      **e**: [letstalkmedicines@gstt.nhs.uk](mailto:letstalkmedicines@gstt.nhs.uk)

### Your comments and concerns

For advice, support or to raise a concern, contact our Patient Advice and Liaison Service (PALS). To make a complaint, contact the complaints department.

**t**: 020 7188 8801 (PALS)      **e**: [pals@gstt.nhs.uk](mailto:pals@gstt.nhs.uk)  
**t**: 020 7188 3514 (complaints)      **e**: [complaints2@gstt.nhs.uk](mailto:complaints2@gstt.nhs.uk)

### Language Support Services

If you need an interpreter or information about your care in a different language or format, please get in touch:

**t**: 020 7188 8815      **e**: [languagesupport@gstt.nhs.uk](mailto:languagesupport@gstt.nhs.uk)

### NHS 111

Offers medical help and advice from fully trained advisers supported by experienced nurses and paramedics. Available over the phone 24 hours a day.

**t**: 111

### NHS Choices

Provides online information and guidance on all aspects of health and healthcare, to help you make choices about your health.

**w**: [www.nhs.uk](http://www.nhs.uk)

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