

What is hydroxyurea?

Sickle cell disease can cause painful crises, chest crises and strokes. It can also affect other vital organs such as the kidneys and lungs, even when the affected person feels well. Hydroxyurea has been shown to decrease the frequency and severity of pain and chest crises, reduce the need for blood transfusions in some patients, and reduce the number of hospital admissions patients may experience. Hydroxyurea has been well studied in patients, including infants, with sickle cell disease for the past 25 years with good results and few side effects. Hydroxyurea is not a cure for sickle cell disease. However, it is the only medicine currently available that can reduce the negative effects of sickle cell disease on the body and potentially increase one's lifespan.

How does hydroxyurea work?

Hydroxyurea works in three ways:

- 1) It increases the body's production of fetal (baby) hemoglobin – this reduces the amount of sickle hemoglobin in the red blood cells.
- 2) It decreases the amount of white blood cells circulating in the body – this improves blood flow by thinning the blood and making it less sticky.
- 3) It increases the water content of the red blood cells, making them less likely to sickle.

Hydroxyurea is mainly eliminated from the body through the urine. While on this medicine, ensure your child drinks lots of fluids to keep the kidneys working well.

Side Effects

As with all medications, hydroxyurea has some side effects. Most side effects of this medication are seen only at high doses, such as those used for treating cancer. The doses used for treating sickle cell disease are much lower, so most sickle cell patients tolerate hydroxyurea without any problems. However be sure to contact your hematology team if your child has any of the following side effects.

Common side effects

Hydroxyurea can lower the amounts of white blood cells and platelets in the body, so call your hematology team immediately if you notice fever, bleeding or bruising.

Usually changes in white blood cells and platelets will be seen in blood work results before they become serious, so it is important to have outside blood work done on time according to the schedule recommended by your hematology team.

Hydroxyurea has also been shown to decrease sperm production in males. This may or may not be reversible after stopping the medication. However when starting hydroxyurea, the possible side effects of the medication must be weighed against the benefits, and hydroxyurea's ability to mitigate the complications of this potentially fatal disease must be considered.

Rare side effects

Changes in skin and nail color, nausea, vomiting, hair loss, fever, and skin rash are more rare side effects. There is the small possibility of liver or kidney damage, but this side effect can be detected and treated early by monitoring blood work regularly.

Precaution

Hydroxyurea can potentially harm a developing fetus, so it is important that sexually active young women taking hydroxyurea also use birth control methods such as the contraceptive pill and/or condoms. If you are considering starting a family, talk with your hematology team before stopping hydroxyurea. If you think you may already be pregnant, stop taking hydroxyurea and call your hematology team as soon as possible.

Note There has been speculation in the past that hydroxyurea may increase a person's risk for developing leukemia. It is important to know that this has not been proven. Research has shown *no difference* in cancer risk between someone with sickle cell disease taking hydroxyurea and someone with sickle cell disease who is not taking hydroxyurea.

How is hydroxyurea taken?

Hydroxyurea is taken by mouth once a day, every day. It comes in both liquid and capsule form. It should be taken on an empty stomach with plenty of water. Some children find it easiest to take it right before bed. Do not give your child more or less than what is specified because that dosage has been determined exclusively for your child. It is very important that the medication be taken every day, even

when your child is sick. Missing a day or two here and there **will** affect how well the medicine works.

Missed Doses

If your child misses a dose, **do not** double the next dose; just continue with the regular schedule. If your child vomits the dose within 30 minutes of taking it, repeat the dose. If it has been longer than 30 minutes, do not repeat the dose but rather continue with the regular schedule the following day.

Monitoring

Your child will start this medication at a low dose, and the dose will be increased every 2 to 3 months until they are able to tolerate the maximum therapeutic dose.

In order to avoid side effects, blood work will need to be done at an outside lab every two weeks until it is stable (this can take up to 3 months). After that it will be done monthly. Be aware that blood work will need to be done more frequently with each dose increase and if changes are noted in the results.

Important Note

This medication can take up to 6 months to work effectively. It is important that you do **NOT** stop the medication without talking to the hematology team, even if you think the medicine is not working. If you feel the medicine is not working, discuss this with your hematology team, as the dose may need to be increased. **The beneficial effects of hydroxyurea are only sustained while it is being taken at the recommended dose.** Please adhere to your doctor's instructions for taking this medication.

For more information contact:

Heather McCartney, BSN RN
Hemoglobinopathies Nurse Clinician, BC Children's Hospital
Office: (604) 875-2345 ext 7103
Pager: (604) 707-3895
Email: hmccartney@cw.bc.ca