

# YOU AND YOUR HYDROXYUREA

## What is hydroxyurea?

Hydroxyurea is a medicine you take once a day that can make your sickle cell disease less severe. Taking it regularly can raise your hemoglobin level and lower the number of times you get pain and acute chest syndrome. This can decrease the number of admissions to the hospital and the number of transfusions you may need. No, it is not a cure but it can improve your quality of life.

## Who should take hydroxyurea?

We recommend hydroxyurea for anyone with sickle cell disease who has had two or more episodes of pain or acute chest syndrome in their lifetime. Your sickle cell team will offer the medication if we feel it could help you, but the final choice is made by you and your family.

## How does hydroxyurea work?

Hydroxyurea works in two ways. The first way is in the red blood cells. Red blood cells contain hemoglobin that carries oxygen to all parts of the body. People who have sickle cell disease make an abnormal hemoglobin called sickle hemoglobin. Sickle hemoglobin causes the red blood cells to become stiff and sticky. The sickle cell can then cause a block in the flow of blood to important organs. Hydroxyurea helps the body make another hemoglobin called fetal hemoglobin or hemoglobin F which is usually only produced by babies in the fetal stages of life (before birth). With higher levels of hemoglobin F, red blood cells are less likely to sickle and cause problems.

The second way that hydroxyurea works is by lowering the number of neutrophils (infection— fighting white blood cells). This can be a good thing, because the increased neutrophil count makes the blood “thicker” and “stickier” which increases the chance that the sickled cells will get stuck in the veins and cause the blockage that causes the problems.

## Is taking hydroxyurea easy?

Yes and no. Hydroxyurea is a medication that only has to be taken once a day. If you are able to swallow pills, it is even easier although liquid is also available. However, taking hydroxyurea is a big commitment. The medication only works if you take it every day so it has to be taken EVERY day. Lab work should be drawn monthly (or more often if needed) to monitor that the neutrophils do not get too low. If the neutrophils get too low, the risk of infection goes up. When this happens, the hydroxyurea has to be held and more labs are needed to determine when is a safe time to restart the medication. There are also doctor visits every 3 months. It is a BIG commitment but for people who have frequent hospitalizations or pain, hydroxyurea can improve your quality of life.

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## **Is hydroxyurea safe?**

Hydroxyurea is safe when prescribed and monitored by doctors who know how to care for patients with sickle cell disease. It may cause a temporary upset stomach, but taking it with food helps. There is also the increased risk for infection when the neutrophil count drops. This risk is minimized by your monthly lab work.

The long-term side effects of hydroxyurea are not fully known. Some people wonder if hydroxyurea could cause cancer if taken for a long time, but this has not been seen in people with sickle cell disease who have been treated for as long as 20 years. Actually, adults with sickle cell disease who take hydroxyurea seem to live longer than those who do not. There is no known limit to how long you can be on the medication so you should take it as long as it is working.

## **How will I know that hydroxyurea is working?**

You will not feel better right away, because it takes a few months for hydroxyurea to start working. If you take it once a day every day, then you will eventually notice that you feel better and have fewer problems like pain. Your doctors can also see changes in your lab work that shows the medication is working. There are a few people who won't respond, so the medication doesn't help. But, you should try the hydroxyurea for at least six months before stopping it.

## **What do I need to know about hydroxyurea and pregnancy?**

People who take hydroxyurea must not get pregnant or father a child. Hydroxyurea is a known teratogen, which means that if you get pregnant or father a child the baby might be deformed. The malformation can range from life limiting to life threatening. If you are of child-bearing age, then you must always use some type of birth control or not have sex at all. If you want to have a baby, you must stop hydroxyurea for several months before getting pregnant. This applies to both men and women as the hydroxyurea can affect the sperm. A mother can start taking hydroxyurea again after the baby is born and breast feeding is completed.

## **Is hydroxyurea the only treatment for sickle cell disease?**

Hydroxyurea is the only non-invasive option at this time. Chronic red blood cell transfusions have been used for many years to treat severe problems of sickle cell disease; however, this introduces the slim but still real chance of transfusion-acquired HIV or Hepatitis C as well as other problems such as iron overload with liver and heart damage. Also, bone marrow or stem cell transplantation (replacing a patient's bone marrow with normal bone marrow) can cure sickle cell disease, but requires a suitable donor and can be associated with many severe complications.