

Hydroxyurea for Sickle Cell Disease

Treatment Information from the



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Introduction

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What problems can sickle cell disease cause?

Sickle cell disease can cause:

- **Anemia** – when you have fewer red blood cells to carry oxygen in your body, which can make you feel tired.
- **Pain crisis** – pain in your chest, stomach, or bones. This happens when sickle cells block your blood vessels.
- **Acute chest syndrome** – a lung problem that happens when sickle cells block the blood vessels in your lungs. This can be life threatening and you will need to go to the hospital.
- **Organ damage** – harm to important organs like your brain, heart, lungs, kidneys, and eyes. This can lead to serious problems like kidney failure or vision loss.

Sickle cell disease can even shorten your life – but hydroxyurea can help you live longer. That's why it's important to consider taking it.



Hydroxyurea can help people with sickle cell disease live longer .

Who should take hydroxyurea?

If you have any form of sickle cell disease, hydroxyurea could help you. This is true even if your symptoms aren't severe.

Different types of sickle cell disease respond to hydroxyurea differently. If you aren't sure which type of sickle cell disease you have, ask your doctor – and say that you'd like to know how hydroxyurea could help you.

- Experts strongly recommend hydroxyurea for people with **sickle cell disease type SS** or **type sickle beta zero (S 0) thalassemia** (“thal-uh-SEE-me-uh”). Hydroxyurea is proven to help people with both of these forms of sickle cell disease.
- If you have **sickle cell disease type SC** or **type sickle beta plus (S +) thalassemia**, talk to your doctor. Hydroxyurea can help, but scientists have done less research on what hydroxyurea can do for people with these types of sickle cell disease.

Hydroxyurea can even help people who rarely have obvious problems caused by sickle cell disease.

What are the types of sickle cell disease?

There are many different forms of sickle cell disease:

- Sickle cell anemia, which includes sickle cell disease type SS and type sickle beta zero (S 0) thalassemia
- Sickle cell disease type SC
- Sickle cell disease type sickle beta plus (S +) thalassemia
- Sickle cell disease type SD, SE, and other sickle cell disease variants

The Research on Hydroxyurea

Research shows that hydroxyurea is safe and helpful for people with sickle cell disease.

The Multicenter Study of Hydroxyurea

In 1992, scientists started a study of 299 people to see if hydroxyurea could help adults with these kinds of sickle cell disease:

- Sickle cell disease type SS
- Sickle cell disease type sickle beta zero (S 60) thalassemia

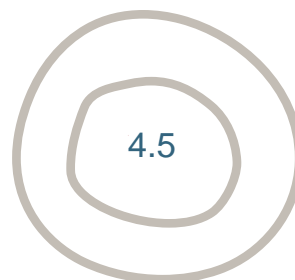
To do this, they randomly put people in the study into 2 groups. One group got hydroxyurea. The other group got a placebo (a pill that has no medicine). The people in the study didn't know which pill they were taking.

What did the study show?

The people who took hydroxyurea had fewer problems caused by their sickle cell disease.

They had half as many pain crises.

- The people who took the placebo had **nearly 5** pain crises in a year.
- The people who took hydroxyurea had about half as many – **fewer than 3** pain crises a year.



4.5 pain crises per year with the placebo



2.5 pain crises per year with hydroxyurea

2.4 hospital stays per year with the placebo

1 hospital stay per year with hydroxyurea

They got acute chest syndrome less often.

- **35** out of every 100 people who took the placebo got acute chest syndrome.
- Only **16** out of every 100 people who took hydroxyurea got acute chest syndrome – that's about half as many.

They needed fewer blood transfusions.

- **50** out of every 100 people who took the placebo got transfusions.
- Just **32** out of every 100 people who took hydroxyurea got transfusions.

They didn't have more side effects.

The researchers also looked at side effects and found that people taking hydroxyurea didn't have any more side effects than people taking the placebo.

Source: Charache, S., Terrin, M. L., Moore, R. D., Fuchs y 1, G3t /T11_ 321 than people taking the placebo.



Safety and Side Effects

Is it safe to take hydroxyurea for many years?

Yes. Many people with sickle cell disease have taken hydroxyurea safely for over 20 years. Even young children can take it.

Does hydroxyurea cause cancer?

No. There's no evidence that hydroxyurea causes cancer in people with sickle cell disease. It's been used safely since the 1980s.

Nana's Story*

“Hydroxyurea has given me the opportunity to be in control of my health and life. ”

Nana started taking hydroxyurea when she was just 12 years old. She'd already been in the hospital many times. After Nana started taking hydroxyurea, she was in the hospital less and less. She even got near perfect attendance at school – and went on to study psychology at the University of Virginia.

*The stories in this booklet are from real people who shared their experiences with ASH.

What side effects can hydroxyurea cause?

All medicines can have side effects. Some people who take hydroxyurea may experience these side effects:

- Thinning hair or mild hair loss
- Fingernail beds that turn darker
- Nausea (feeling sick to your stomach)

Very rarely, hydroxyurea can cause more serious side effects. But most people with sickle cell disease who take hydroxyurea don't have any serious side effects.

If you have any new symptoms after you start taking hydroxyurea, tell your doctor – you may be able to take a lower dose.



Most people with sickle cell disease who take hydroxyurea have few or no side effects. Most side effects are mild.



Will I be able to start a family?

If you're thinking about having a baby, be sure to talk to your doctor about the pros and cons of taking hydroxyurea. Experts are still learning about how hydroxyurea affects your ability to have a healthy baby. Taking it during pregnancy is a personal choice that your doctor can help you make.

Women

- If you are pregnant or planning to get pregnant, talk to your doctor to make a plan.
- Hydroxyurea may increase the risk of birth defects, but we don't know for sure yet.
- Some women choose to stop taking hydroxyurea early in their pregnancy and then start it again during the third trimester (after 29 weeks).

Men

- Hydroxyurea can lower your sperm count, which may already be low due to sickle cell disease.
- Painful erections are a complication of sickle cell disease that can cause permanent damage to the penis. Hydroxyurea may make these painful erections less likely.



Many men and women who have taken hydroxyurea have had healthy babies.

Nikita's Story*

"My baby is perfectly healthy and doing just ne. "

Nikita started taking hydroxyurea after she got sick during a business trip, and it helped her a lot. After taking it for over 5 years, Nikita got pregnant.

She talked to her doctor about continuing to take hydroxyurea, and together they decided the best choice for her and the baby was to stop taking it during her pregnancy. After she gave birth to a healthy baby boy, Nikita started taking hydroxyurea again. Now, both she and her son are doing well.

*The stories in this booklet are from real people who shared their experiences with ASH.





Hydroxyurea isn't very expensive, and most health insurance plans cover it.



What if I take hydroxyurea and it doesn't seem to help?

- Don't be discouraged! Hydroxyurea takes time to work. Try to stick with it for at least a year. It may take that long for your doctor to figure out the right dose for you.
- If you're having side effects, don't stop taking hydroxyurea — talk to your doctor first. Some side effects will go away after you take it for a while.

Adrienne's Story*

"It took a while to start working, but I feel much better now."

For a while, Adrienne was very sick and her blood counts were always low. Her doctor prescribed hydroxyurea for her sickle cell disease, but she stopped taking it after 4 months because she was worried about side effects.

When her doctor explained how hydroxyurea could help her over time, she decided to try it again. She started to feel better after about 6 months. She was able to be more active and she's been in the hospital a lot less.

*The stories in this booklet are from real people who shared their experiences.

The Next Step

Hydroxyurea can help people with sickle cell disease have fewer pain crises and better health. If you have sickle cell disease, it can be helpful. We hope this booklet has answered some of your questions about this treatment.

Think hydroxyurea might be right for you? Start the conversation with your doctor.

Notes
