

# Hydroxyurea for Sickle Cell Disease

Treatment Information from the  
American Society of Hematology





## About the American Society of Hematology

For more than 50 years, the American Society of Hematology (ASH) has been committed to helping hematologists conquer blood diseases. With more than 17,000 members from nearly 100 countries, ASH is the world's largest professional society of hematologists. We're dedicated to furthering the understanding, diagnosis, treatment, and prevention of disorders affecting the blood. To learn about how ASH is working towards conquering sickle cell disease, visit [www.hematology.org/scd](http://www.hematology.org/scd).

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## About This Booklet

This booklet has been prepared by the American Society of Hematology and provides general information about hydroxyurea, which is one of the few FDA-approved therapies to treat sickle cell disease. This booklet is purely an informational resource. It does not provide medical advice and is not intended to substitute for consultation with a medical professional. People with sickle cell disease should talk to their doctor before making any changes to their treatment.

# Introduction

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If you have sickle cell disease, you know the impact it can have on your life. Sickle cell disease can cause periods of intense pain (called “pain crises”) and other problems. You may even end up in the emergency room or the hospital.

But here’s some good news: a medicine called hydroxyurea (“hi-drox-ee-ure-EE-a”) can help adults and children with sickle cell disease. This medicine can help you have fewer pain crises – and even live longer. Hydroxyurea has helped many people and it could help you.

The American Society of Hematology developed this booklet to educate people with sickle cell disease about this important treatment option. We encourage you to talk with your doctor about your personal treatment plan and whether hydroxyurea could help. The decision to take hydroxyurea is up to you.



## Sickle Cell Disease Basics

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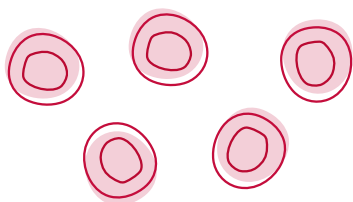
### What is sickle cell disease?

Sickle cell disease is a blood disease that causes problems with red blood cells.

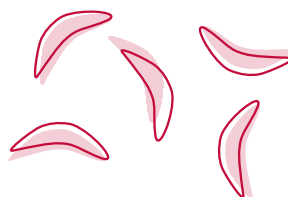
Normally, red blood cells are round and flexible, which allows them to move easily through your blood vessels. Red blood cells contain a protein called hemoglobin that carries oxygen throughout your body.

People with sickle cell disease don't have regular hemoglobin in their red blood cells. Their hemoglobin can give their red blood cells a curved shape, like a banana or a sickle (a farming tool with a curved edge). These sickle cells are hard and sticky, which means they can block the flow of blood in your body and cause serious problems.

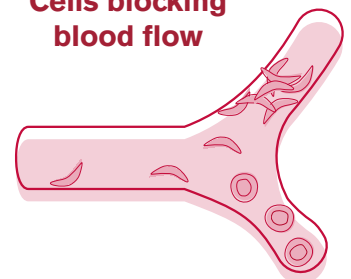
**Normal red blood cells**



**Sickle-shaped cells**



**Cells blocking blood flow**





## 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 Facts

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Hydroxyurea is a medicine that doctors have used to treat people with sickle cell disease since the 1980s. The Food and Drug Administration (FDA) approved it for treating adults with sickle cell disease in 1998. In 2017, the FDA approved it to treat children with sickle cell disease.

Hydroxyurea is also used to treat cancer. But doctors use a lower dose (amount) to treat sickle cell disease than to treat cancer.



**Hydroxyurea can help people with sickle cell disease live longer.**

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## How can hydroxyurea help with sickle cell disease?

Hydroxyurea reduces the problems that sickle cell disease causes. People with sickle cell disease who take hydroxyurea have fewer:

- Pain crises
- Episodes of acute chest syndrome
- Blood transfusions
- Hospital stays

Hydroxyurea can also prevent or slow down damage to your organs.

### Cole's Story\*

**“This was my first activity without pain in years.”**

Before Cole started taking hydroxyurea, she had frequent pain crises. For a while, she was in the hospital every few weeks. She wasn't able to keep a job.

Then Cole's doctor recommended hydroxyurea. After she started taking it, Cole began to feel better. She even walked a 5K race just a few months later without going into crisis or having any pain. She was able to start working again – and she has gone more than 4 years without a major pain crisis.

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

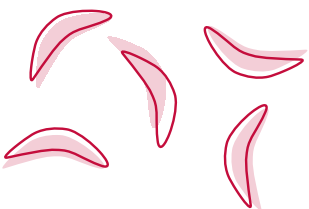


## How does hydroxyurea work?

Hydroxyurea makes your red blood cells bigger. It helps them stay rounder and more flexible – and makes them less likely to turn into a sickle shape.

The medicine does this by increasing a special kind of hemoglobin called hemoglobin F. Hemoglobin F is also called fetal hemoglobin because newborn babies have it. When you have higher levels of hemoglobin F, your red blood cells are less likely to cause problems.

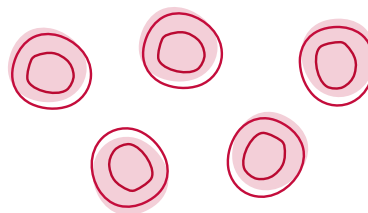
### Sickle-shaped cells



Use of hydroxyurea  
over time



### Normal, round red blood cells





## 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 $\beta$ 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 $\beta$ +) 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 $\beta$ 0) thalassemia
- Sickle cell disease type SC
- Sickle cell disease type sickle beta plus (S $\beta$ +) 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 (Sβ0) 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?

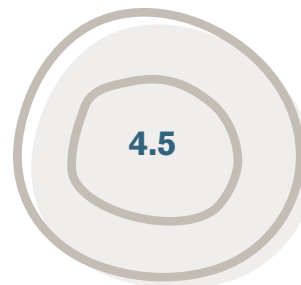
The people who took hydroxyurea had fewer problems caused by their sickle cell disease.

### 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.

### They had fewer hospital stays.

- People who took the placebo had **more than 2** hospital stays a year due to pain crises.
- People who took hydroxyurea had **only 1** hospital stay a year because of a pain crisis.



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

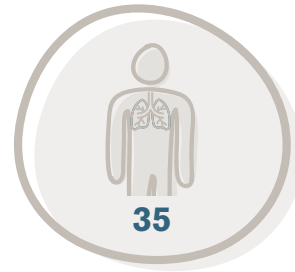


1 hospital stay per year with hydroxyurea

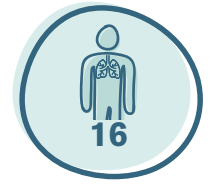


## They got acute chest syndrome less often.

- **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.



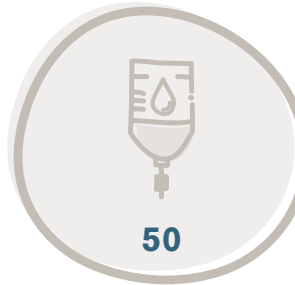
**35 of 100 people got acute chest syndrome with the placebo**



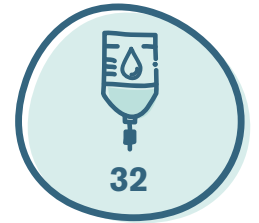
**16 of 100 people got acute chest syndrome with hydroxyurea**

## 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.



**50 of 100 people got transfusions with the placebo**



**32 of 100 people got transfusions with hydroxyurea**

## 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., Dover, G. J., Barton, F. B., Eckert, S. V., McMahon, R. P., and Bonds, D. R. (1995). Effect of hydroxyurea on the frequency of painful crises in sickle cell anemia, *The New England Journal of Medicine*, 332(20), 1317-1322. <http://doi.org/10.1056/NEJM199505183322001>

## What have other studies shown?

Many other studies have shown that hydroxyurea helps people with sickle cell disease – even children. One study showed that hydroxyurea is helpful and safe for children as young as 9 months old.



# Safety and Side Effects

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## 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.**

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## 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 fine.”

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.



# Taking Hydroxyurea

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## What do I need to know about taking hydroxyurea?

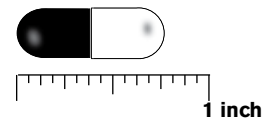
- Most people take hydroxyurea pills once a day. Your doctor will prescribe the dose he or she thinks is right for you. Sometimes you may need to take a different number of pills on certain days.
- Hydroxyurea is safe to take with most other medicines – but it's always a good idea to check with your doctor or pharmacist before starting a new medicine.
- Hydroxyurea pills are capsules that are about three-quarters (3/4) of an inch long.



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

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**Actual size of sample hydroxyurea pill**



## Checking your blood count

You'll need to get your blood cell counts checked regularly when you take hydroxyurea. When you first start taking it, you may need to get your blood counts checked every month.

Ask your doctor about your blood count numbers to see how they change. The changes in your blood counts can be a good sign that the hydroxyurea is working!



**When taking hydroxyurea, getting regular blood counts helps your doctor see how the medicine is working.**

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## These tests will check for:

- **Hemoglobin**, the protein that carries oxygen in red blood cells. Hydroxyurea works by making your hemoglobin level **go up**.
- The **size of your red blood cells** (measured as “mean cell volume,” or MCV). Hydroxyurea works by making your red blood cells **bigger**.
- **Neutrophils**, a type of white blood cell. Hydroxyurea makes the number of neutrophils **go down**. This is okay as long as your white blood cell count doesn't get too low.

Your doctor might change your dose based on your blood cell counts. For example, if the neutrophils in your blood don't go down, your doctor might increase your dose.

Your doctor will also check to see if some types of blood cells get too low. If this is the case, your doctor might ask you to stop taking hydroxyurea for a while.





## What if I miss a dose?

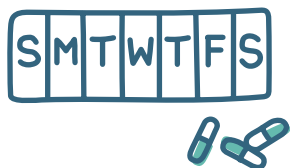
- Hydroxyurea will only work if you take it every day.
- Missing a dose is not dangerous and will not reverse the benefits of the drug.

## Remember to take it every day

It can be hard to remember to take hydroxyurea every day. Here are some tips to help you remember:



Mark your calendar every day after you take it.



Get a pill box with a section for each day of the week. Fill it on the same day every week.



Set an alarm on your cell phone that goes off every day.



Ask friends or family members to help remind you.

## Ndiogou's Story\*

### “Talk to your doctor about giving it a try.”

Ndiogou started taking hydroxyurea when he was 21 years old and noticed the difference a few months later. At first, he had a lot less pain. Eventually, it went away completely.

Sickle cell disease hadn't stopped Ndiogou from playing sports or taking part in other activities, and taking hydroxyurea made life easier for him.

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



## 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, Adrienne decided to try it again. She started to feel better after about 6 months. Now she's able to be more active and she's been in the hospital a lot less, too!

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



## 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 could help you. We hope this booklet has answered some of your questions about this treatment option.

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



# Notes

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