A CHILD'S JOURNEY

Learning about atypical hemolytic uremic syndrome (aHUS)

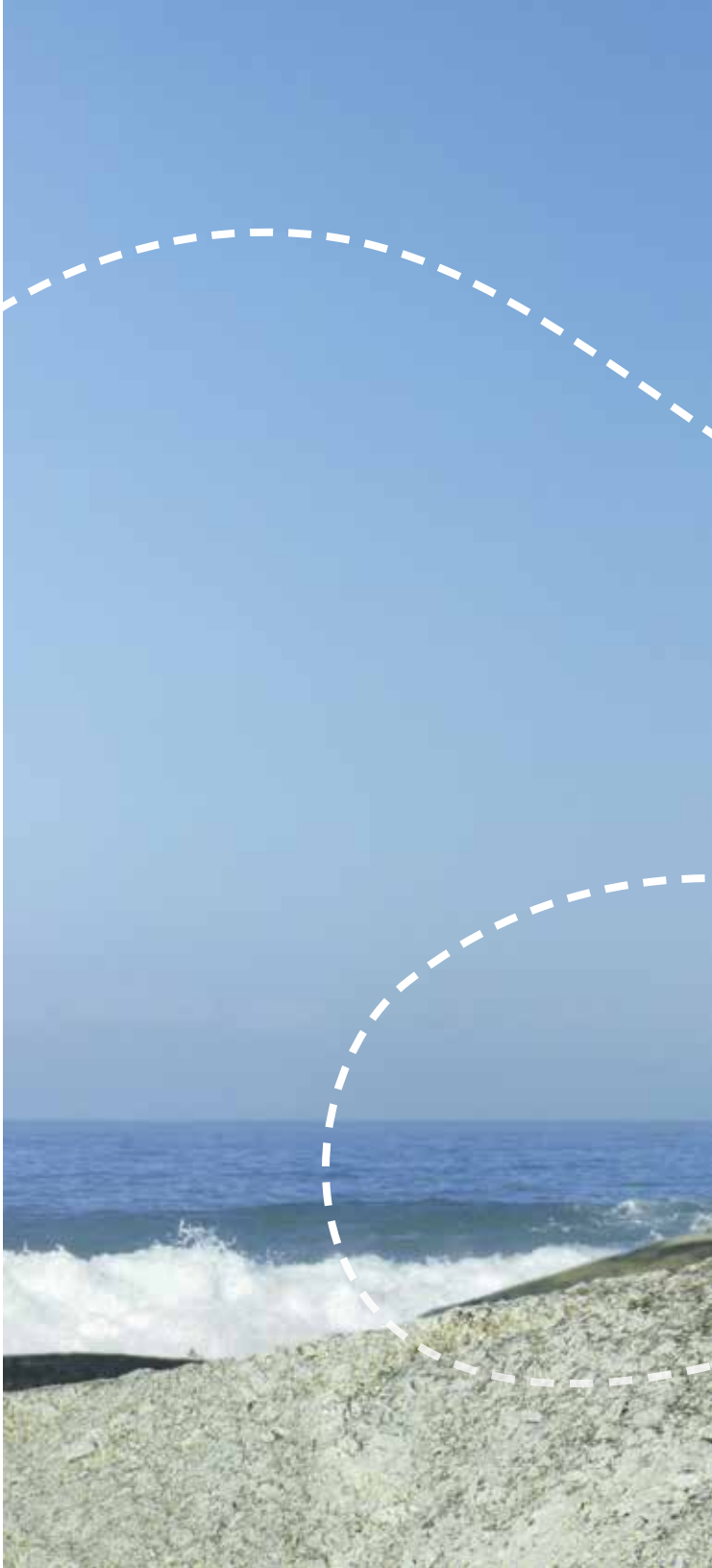
ALEXION®

Let's explore what aHUS is together

Your doctor has just told you that you have **atypical hemolytic uremic syndrome**, or **aHUS**. You are probably feeling a little scared and may have a lot of questions. This booklet is designed to answer some of those questions and help you learn about aHUS.

You are not alone in your journey with aHUS. Use this booklet with your family members, doctors, and nurses to learn more about aHUS.

*Some of the words used when talking about aHUS may be new to you. They are written in **bold** or orange text, and their definitions can be found toward the back of this brochure.*



Let's get started

A CHILD'S JOURNEY

Exploring aHUS together

The journey begins: What is aHUS?

Your doctor has probably told you that aHUS is a very serious disease. You may be wondering what this disease really is and how it will affect you. The first thing you should know is that your doctor will do his or her best to help you manage your aHUS.

aHUS is a lifelong disease that affects your body's blood system.[1]

BLOOD

Your ***blood*** is a special part of your body with many jobs. It looks just like a red liquid, but is actually full of many different parts like cells, proteins, and a liquid called plasma. Blood travels around your body in tubes called vessels. If you break one of those tubes, you may see blood–for example, when you scrape your knee.

Normally your blood flows through your blood vessels, from your heart out to the rest of your body and back again. Your blood brings things like oxygen, nutrients and energy to all the different parts of your body, and takes away things that your body doesn't need anymore.

With aHUS, the small vessels that carry blood around your body can get blocked.[2–5] When blood doesn't flow smoothly, or if it doesn't flow at all to your organs, the organs may not work correctly and this can make you feel sick.[2,4]

What happens when someone has aHUS?

You may already have felt sick because of aHUS. You may have had a stomach ache or a headache, or noticed red or brown **urine** when you went to the bathroom.[1,3,6,7] If you have any of the following signs and symptoms of aHUS, be sure to tell your parents, teacher, or an adult right away:

- Feeling a little confused
- Having trouble breathing
- Feeling tired
- Stomach ache
- Vomiting
- Having diarrhea

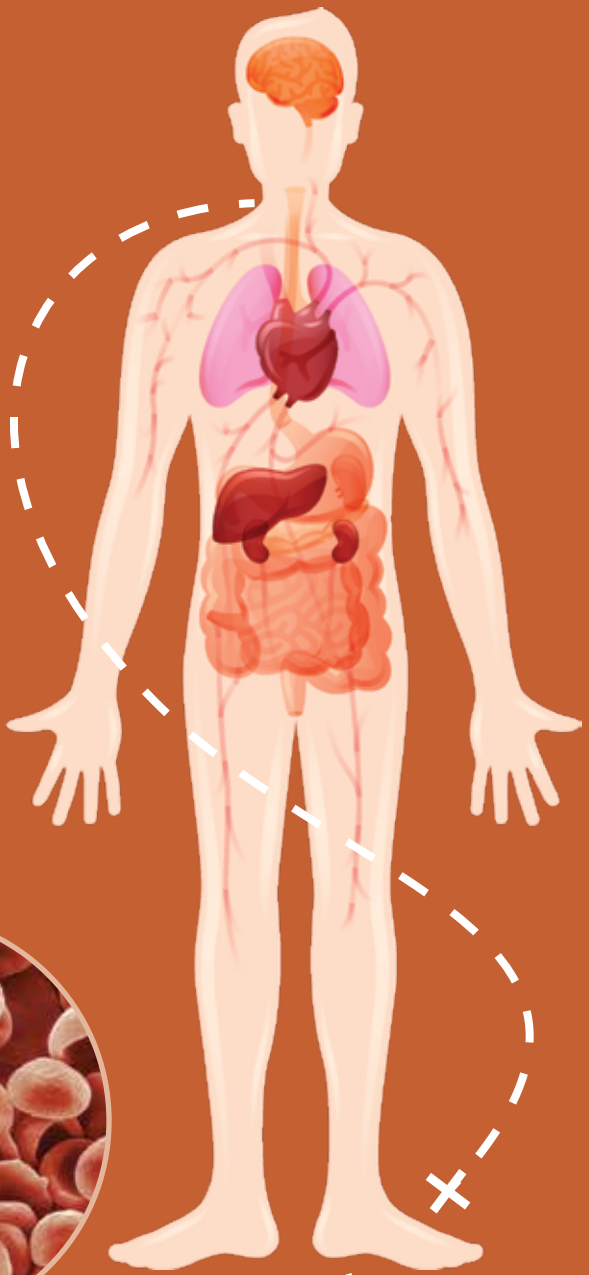
Now that your doctors know you have aHUS, there are things they can do to help you feel better.[4]

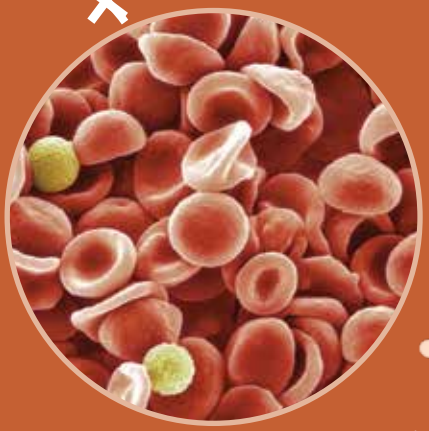
What causes me to feel sick when I have aHUS?

Your blood carries lots of things through your body. One important thing your blood carries is **platelets**. Platelets are tiny parts of the blood that help stop bleeding when you have an injury, like a cut or a bruise.[2] Platelets work by clumping together to form **clots**.

Another part of your blood, called **complement**, helps to fight infection.[2] In people with aHUS, complement is overactive, and this makes your platelets form clots when they shouldn't.[2,3,8] Once clots form inside your small blood vessel walls, blood can't travel around your body the right way. These clots can cause damage to your kidneys, heart, brain, and other organs.[2,9]

A person with aHUS and overactive complement might get very sick and often needs care in a hospital by special doctors to try and fix the damage and save or even replace the organs that are hurt. It's important that you and your doctor work together to help you manage your disease.

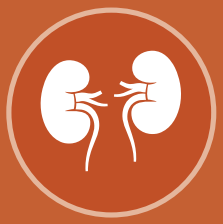




What parts of your body can be affected by aHUS?

BRAIN

Your brain is the part of your body that controls how your organs and muscles move and work. It's the boss of your body!



KIDNEYS

Your kidneys make urine and help get rid of things from your blood that your body doesn't need anymore.

HEART

Your heart is in your chest, and it pumps blood through your body. Your nurses and doctors may listen to your heart using something called a **stethoscope**.

STOMACH AND INTESTINES

Your stomach and intestines digest the food you eat and turn it into energy for your body. When you feel full after eating, it's because your stomach is full of food!

Visit to the doctor: Getting a checkup

Because you have a lifelong disease, you will need to visit your doctor and get your blood tested more often. Checkups help your doctors and nurses learn more about what is going on inside your body. When you go to the doctor, he or she will listen to your heart and lungs by using a stethoscope.

Your doctor might look at your eyes and ears and throat. A nurse may take your temperature with a **thermometer**, which your parents may have done when you were sick and had a fever. A nurse may put a cuff on your arm and puff it up so it hugs your arm—this is called a **blood pressure cuff**. A blood pressure cuff tells the doctor how blood is moving in your body. Below are some terms and definitions that you may hear at the doctor's office.

CHECKUP CHALLENGE

Can you match the terms on the left to their definitions on the right?

1. BLOODWORK	A	is part of your blood that helps to fight infection.
2. DIALYSIS	B	is a doctor who takes care of kids.
3. HEMATOLOGIST	C	is a doctor who specializes in studying and treating your kidneys.
4. COMPLEMENT	D	is a doctor who specializes in studying and treating your blood.
5. NEPHROLOGIST	E	when a doctor draws your blood to test what is in it.
6. PEDIATRICIAN	F	is a process that doctors use to clean your blood when your kidneys are not working well.

ANSWERS

1. E; 2. F; 3. D; 4. A; 5. C; 6. B

Questions you may think of along the way

What will happen when I go back to school?

You may have missed some school while you were sick. Your health care team can help you work with your school to get caught up. More importantly, they are there if you need to talk to someone.

Who should I tell if I feel sick again?

You should tell an adult right away if you don't feel well. Talk to your parents or a teacher if you feel sick.

How often do I have to visit my doctor?

Because aHUS doesn't go away,[10] you will probably visit your doctor often. You may also visit new doctors. Your doctors will work with you to help you stay healthy.

Will I always have aHUS?

Yes, because the gene defect that causes aHUS does not go away. You and your doctor, as a team, will work together to manage your aHUS.

Who gets aHUS?

Anyone can have aHUS—a boy or girl, a kid or an adult. aHUS is a rare disease.[2,5,8] This means that you and your family may not know anyone else with aHUS. You may want to learn more about other kids who have aHUS. They probably are having some of the same feelings you are having.

Continuing your journey:

You may feel differently now that you know you have aHUS; but, you are still the same kid you were before. It's normal to be sad, mad, happy, or confused. Take some time to figure out who you can talk to when you have questions, if you don't feel good, or if you are upset. It's important for you to remember that many people want to help you feel better.

Use the space below to draw a picture of how you feel, which you can share with your family members, doctors, and nurses. This may help people you know understand how you are feeling inside, so they can help you.

Use this space to draw how you are feeling, or to show what part of your body is bothering you.

Go ahead, fill in your answers.

Who can you talk to when you are at home?

Who can you talk to when you are at school?

When you have questions about aHUS, talk to your doctor or nurse.

Your doctor's name is:

Your nurse's name is:

When you are not feeling like yourself, what cheers you up?

What can you bring to the doctor's office to make the time pass more quickly?

Support groups: Places to learn more

More information about aHUS can be found online. Remember to ask your parents before going online.

aHUSSource
atypical Hemolytic Uremic Syndrome (aHUS)

aHUS Source

Provides patients and caregivers information on aHUS, along with a guide to help you and your doctor work together to manage aHUS. You can also access OneSource™, where you can speak witha registered nurse to ask questions about aHUS.

Website: www.ahussource.com

Genetic and Rare Diseases Information Center (GARD)

Helps people find information about genetic and rare diseases. Information Specialists are available to talk with you about aHUS in both English and Spanish.

Website: www.rarediseases.info.nih.gov/GARD

The Atypical HUS Foundation

A volunteer organization open to patients, family, friends, caregivers, researchers, and medical personnel. The Foundation encourages patients and researchers to share information and their personal experiences to foster a better understanding of aHUS. The overall goal is to gather people together to improve the lives of patients and families dealing with aHUS.

Website: www.atypicalhus.ning.com

The Global Genes Project

An organization that works to meet the needs of people living with rare diseases. This organization aims to build awareness of rare diseases and to provide resources and connections to patients and their families. The Global Genes Project hosts meetings for patients with aHUS and their families across the country.

Website: www.globalgenes.org

National Organization for Rare Disorders (NORD)

NORD is dedicated to helping people with rare, or "orphan," diseases. This organization helps patients access assistance programs for medication. They host meetings for patients with aHUS and their families to help them connect to others and provides information on rare diseases by sharing patients' personal stories.

Website: www.rarediseases.org

Support for people living with aHUS

OneSource is a personalized program that provides education, case management, and support for patients and their caregivers. OneSource is staffed by Alexion Nurse Case Managers, all of whom are registered nurses with extensive clinical experience.

Website: www.ahussource.com/Patient/OneSource

Definitions: New words you may hear

Atypical hemolytic uremic syndrome (aHUS) is an illness that affects your blood and blood vessels.[2] aHUS can make you feel sick when your blood can't travel to all the different parts of your body.[2]

Blood flows through your body in small tubes called blood vessels. It carries important things, like energy and nutrients, to all your body parts.

Blood pressure cuff is a tool your doctor may use to see how your blood is flowing through your blood vessels. It will wrap around your arm and feel nice and snug.

Clot is blood that has been converted from liquid to solid state, blocking flow of blood through your blood vessels. A clot can block blood from getting to where it needs to go.

Complement system is a natural part of the immune system that helps protect the body from foreign substances like bacteria or other infections.[2] The complement system is made up of a group of proteins found in the blood.[2]

Creatinine is a waste product from your muscles. Usually, your kidneys work to get rid of creatinine from your body by putting it in your urine. If you have too much creatinine in your blood, it might mean your kidneys are not working the right way.

Genetic disease is a type of illness that you were born with. There is nothing that you did to cause aHUS. You will always have aHUS; however, you and your doctors will work together to keep you feeling good.

Platelets (or thrombocytes) are blood cells whose function is to help the blood clot to stop bleeding.

Stethoscope is a tool your doctor will use to listen to your heart.

Thermometer is a tool your doctor will use to take your body temperature.

Urine is a liquid that comes out when you go to the bathroom. This is how your body gets rid of things it doesn't need anymore.

References

1. Noris M, Caprioli J, Bresin E, et al. Relative role of genetic complement abnormalities in sporadic and familial aHUS and their impact on clinical phenotype. *Clin. J. Am. Soc. Nephrol. CJASN* 2010;5(10):1844-1859. **2.** Noris M, Remuzzi G. Atypical hemolytic–uremic syndrome. *N. Engl. J. Med.* 2009;361(17):1676-1687. **3.** Loirat C, Fremeaux-Bacchi V. Atypical hemolytic uremic syndrome. *Orphanet J. Rare Dis.* 2011;6:60. **4.** Nester CM, Thomas CP. Atypical hemolytic uremic syndrome: what is it, how is it diagnosed, and how is it treated? *ASH Educ. Program Book* 2012;2012(1):617-625. **5.** Geerdink LM, Westra D, Wijk JAE van, et al. Atypical hemolytic uremic syndrome in children: complement mutations and clinical characteristics. *Pediatr. Nephrol.* 2012;27(8):1283-1291. **6.** Ažukaitis K, Loirat C, Malina M, Adomaitiene I, Jankauskiene A. Macrovascular involvement in a child with atypical hemolytic uremic syndrome. *Pediatr. Nephrol.* 2014;29(7):1273-1277. **7.** Neuhaus TJ, Calonder S, Leumann EP. Heterogeneity of atypical haemolytic uraemic syndromes. *Arch. Dis. Child.* 1997;76(6):518-521. **8.** Kavanagh D, Goodship TH, Richards A. Atypical hemolytic uremic syndrome. *Semin. Nephrol.* 2013;33(6):508-530. **9.** Zipfel PF, Heinen S, Skerka C. Thrombotic microangiopathies: new insights and new challenges. Curr. *Opin. Nephrol. Hypertens.* 2010;19(4):372-378. **10.** Laurence J. Atypical hemolytic uremic syndrome (aHUS): making the diagnosis. *Clin. Adv.* Hematol. Oncol. 2012;10(10 Suppl 17):1-12.

Discovering more: Your notes and questions

Some kids find that it helps to take notes when they talk to their doctors, nurse case managers, and others. Use this section to write down anything you want to remember for later.