GET TO KNOW aHUS

A COMPREHENSIVE GUIDE TO EVERYTHING YOU NEED TO KNOW

ALEXION®
AstraZeneca Rare Disease

All the answers to your questions are here

Atypical hemolytic uremic syndrome (aHUS) is a lifelong disease that occurs when your body is unable to control part of your immune system. Learning that you or a loved one has been diagnosed with aHUS can be a difficult experience, but know that you are not alone. With the right tools and support, you can feel confident as you move forward and take control of your health and your life. Understanding the disease is the first step.

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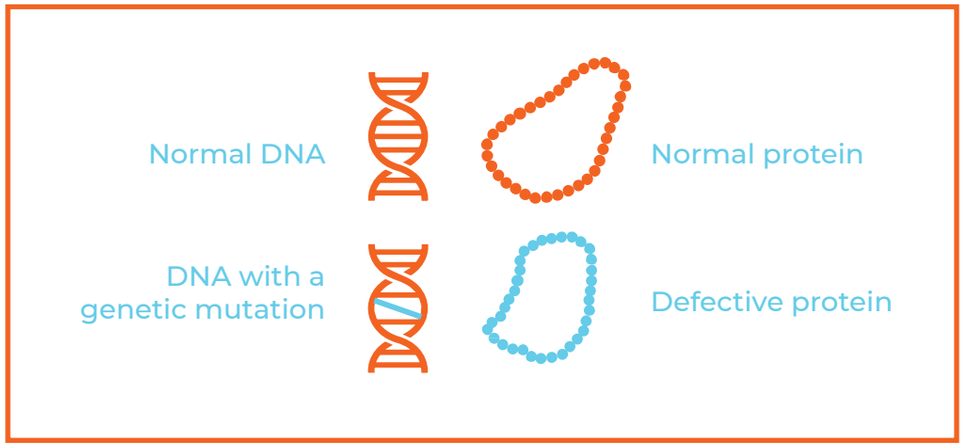
What causes aHUS?

aHUS is a genetic disease, which means that it is caused by a genetic mutation.

Genes are coded in DNA, and are blueprints or instructions for the body to make proteins. A genetic mutation is when genes undergo permanent changes that cause them to have the wrong instructions.



When the genes involved in aHUS are changed, certain proteins are either not formed or don't work properly and are unable to regulate a part of your immune system called the "complement system."

There are several genes involved in aHUS, with more still being discovered through research. You may wish to have genetic testing done to help you better understand the causes of your aHUS and inform your family members who may share those genes.

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Understanding the immune system

The main role of your immune system is to protect your body from foreign (or outside) substances like viruses and bacteria.

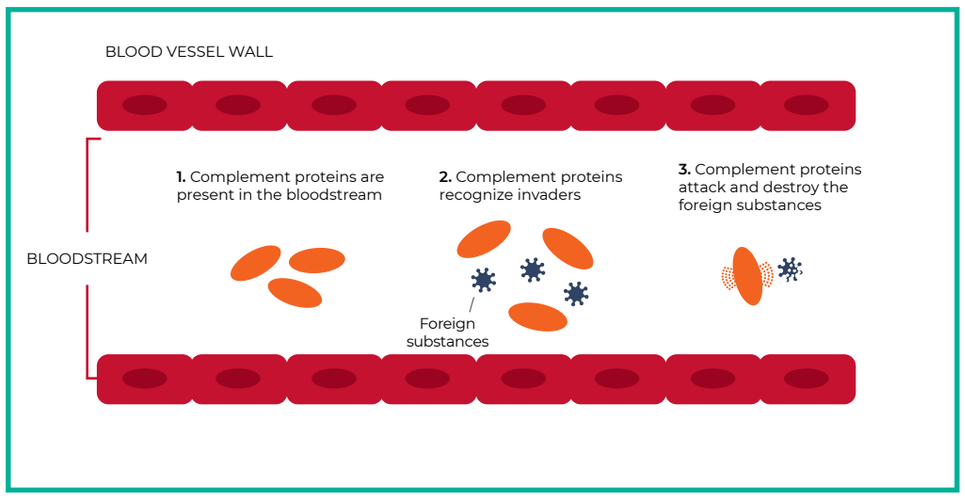
The complement system

“Complements proteins” are a group of proteins that are part of your immune system. Proteins are molecules in your body that have many different functions, including protecting you from foreign substances.

Complement proteins are responsible for recognizing, attacking, and destroying foreign substances that could harm you. They are always present in the blood stream—ready to attack foreign substances and to defend the body from harm. The complement system is carefully controlled so that it targets only foreign substances and not the body’s own healthy cells.

This is all part of a normal process in your body called the “immune response.”

NORMAL IMMUNE RESPONSE



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What is aHUS?

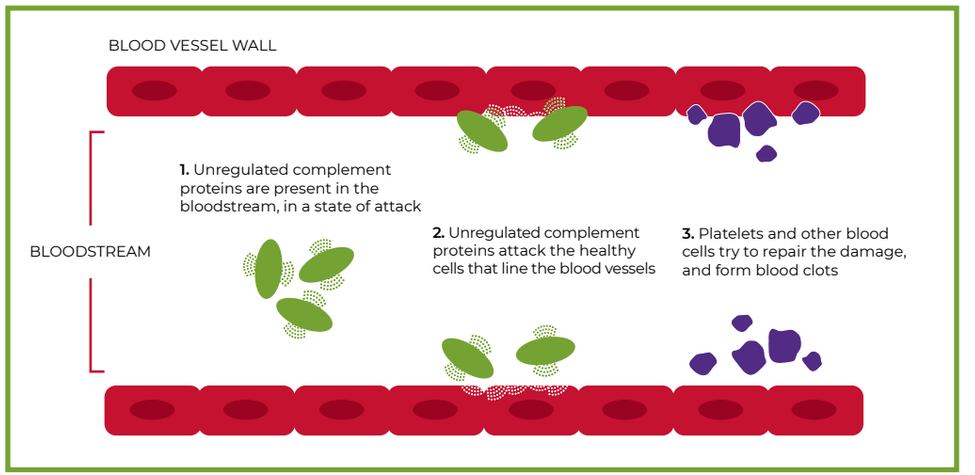
Now that you have a better understanding of the immune system, let's explore what happens when you have aHUS.

An unregulated complement system

In people with aHUS, the complement system is not properly regulated and is continuously in a state of attack—even when your body has not come into contact with a foreign substance.

In this state, the complement system begins to attack **healthy** cells that line the blood vessels in your body, not just foreign substances.

aHUS IMMUNE RESPONSE



Why are blood clots dangerous?

When unregulated complement proteins attack the healthy cells of the blood vessel walls, these cells become damaged and swollen. To try and repair the damage, the body recruits platelets and other blood cells to the site of attack. These cells stick together and form blood clots within your blood vessels.

Blood clots can block normal blood flow to some very important organs—which can damage them and affect how well they function. If not treated, this damage can be irreversible and lead to permanent loss of organ function.

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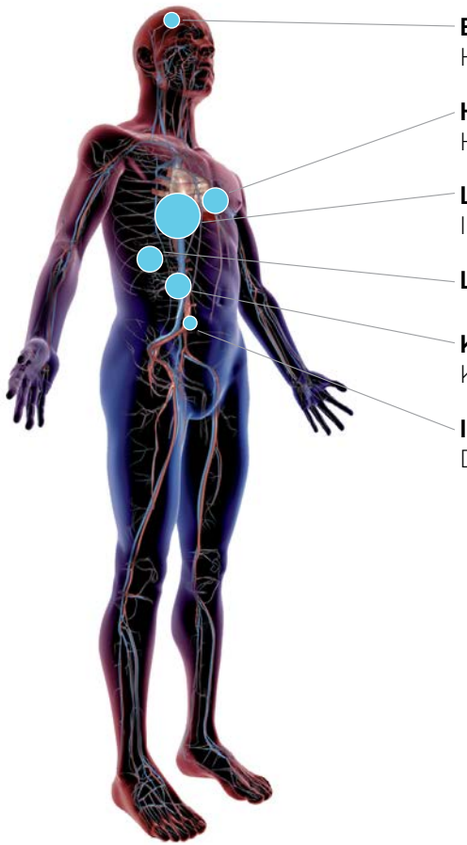
What are the risks of having aHUS?

The blood clots from aHUS can block normal blood flow to various organs in the body—which can damage them and affect how well they function.

KEY ORGANS AFFECTED BY AHUS



Brain
Headaches, seizures, stroke, coma

Heart
High blood pressure

Lung
Infection, bleeding, fluid in the lungs

Liver

Kidneys
Kidney disease (mild or severe)

Intestines
Diarrhea, bleeding

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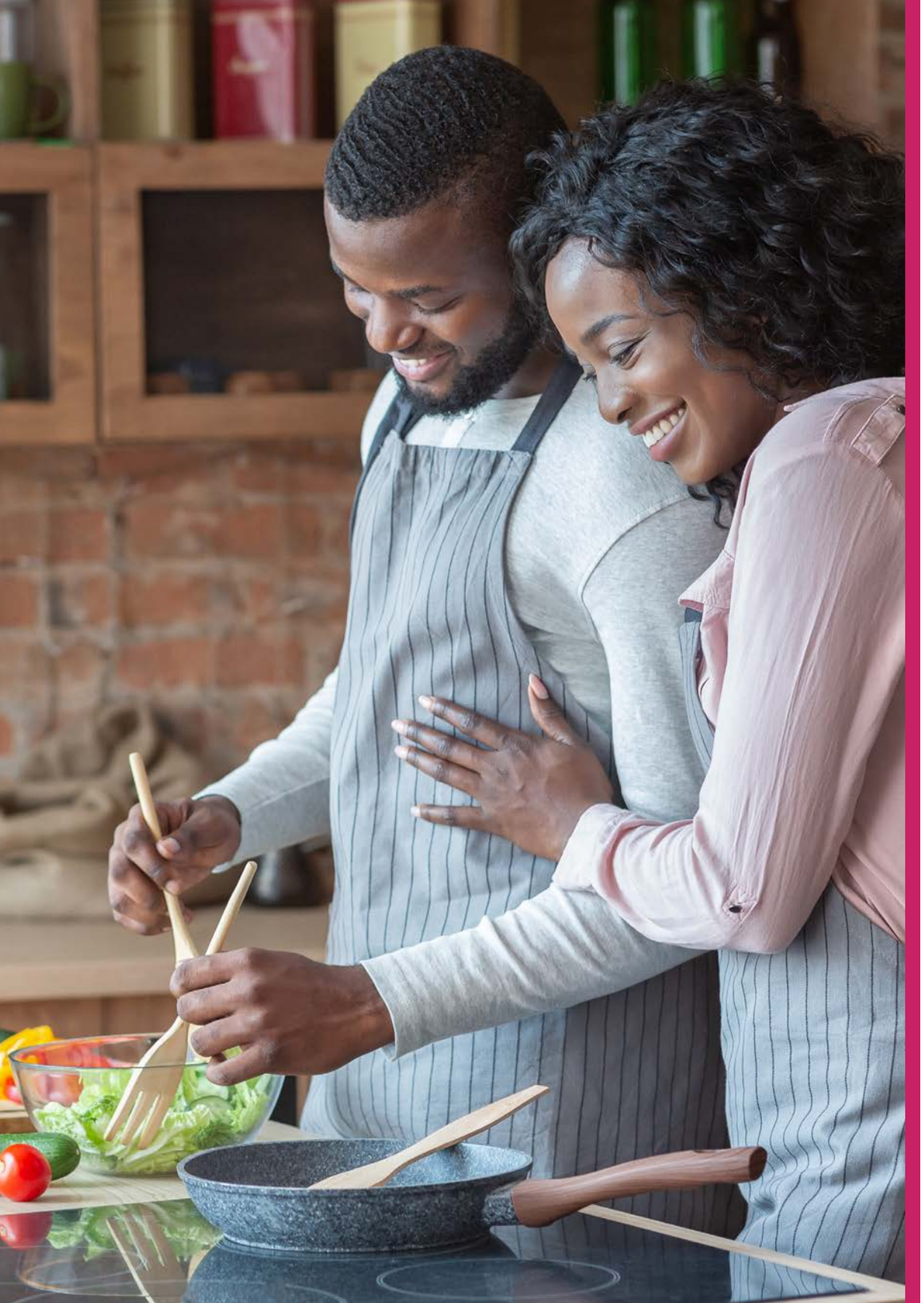

What to look for with aHUS

aHUS can be different for everyone, and managing it means learning to identify the common signs and symptoms that you may experience. You may not always experience the symptoms or see the signs of aHUS, but they include:

Nausea and vomiting

Confusion

Shortness of breath

Stomach pain

Diarrhea

Fatigue

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Working with your healthcare team

With a rare disease like aHUS, it's important for you to work closely with your healthcare team so that you can take control and prevent the complications associated with this disease. Your doctor will monitor your progress to help you determine when you are ready to start treatment and what treatment option is right for you.

COMMON BLOOD TESTS WHEN YOU HAVE aHUS

- **Hemoglobin & hematocrit**
  Related to the ability of the blood to carry oxygen
- **Platelets**
  Help with the blood clotting process
- **White blood cells (WBCs)**
  Part of the immune system that defend against infection
- **Lactate dehydrogenase (LDH)**
  A chemical marker of high aHUS activity
- **Haptoglobin**
  A marker of ruptured (broken open or "popped") blood cells
- **Creatinine**
  A measure of how well the kidneys are working
- **Blood urea nitrogen (BUN)**
  A measure of how well the kidneys are working

Tips to make the most of your doctor's appointments

- Bring notes to your appointments with any concerns you may have
- Ask your doctor about how aHUS can affect your body and how to prevent permanent damage to your organs
- Tell your doctor how you have been feeling and if there have been any changes in your health
- Ask your doctor about your lab results in order to find out what they mean
- Inform your doctor about all medications that you're currently taking (including herbal and other natural remedies)
- Be sure to let your doctor know if you have plans to travel—you may have to take certain considerations into account
- Ask lots of questions!

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Remember, your healthcare team is here to help you—don't be afraid to have an honest and open discussion!

What can be done about aHUS?

While aHUS is a serious disease, there are treatments available. It's important that you and your doctor talk about your treatment options and determine what treatment is right for you.

Blood transfusions

This can be a first therapy that people with aHUS receive. If you have anemia (low red blood cells), you may receive a blood transfusion in the hospital. This does not treat the underlying disease of aHUS but can help return the blood to a more normal level for a time.

Plasma therapies

Plasma is the liquid part of the blood that carries blood cells. There are two different plasma therapy options for treating aHUS:

- In *plasma infusion*, plasma from healthy donors is transfused into a patient with aHUS.
- In *plasma exchange* (or plasmapheresis), blood is taken from an aHUS patient, the damaged plasma in the blood is replaced with healthy donor plasma, and then the blood is transfused back into the patient.

Neither plasma therapy option is a treatment for the underlying disease of aHUS, but they can help return the blood to a more normal level for a time.

Dialysis

Because aHUS can damage the kidneys and affect how well they function, some people with aHUS may require dialysis. In kidney dialysis, a special machine performs the function of the kidneys by removing waste and excess water from the blood. This does not treat the underlying disease of aHUS but is important if the kidneys are too damaged to function properly on their own.

Medications

Targeted therapy can help prevent the unregulated complement system of your immune system from attacking the walls of your blood vessels. It provides significant benefits, including reducing the severity of symptoms and the need for transfusions, and improving anemia.

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Getting help along the way

Your healthcare team

Together, you and your doctor and nurses can develop a plan to treat your aHUS and watch your progress. Your healthcare team is not just a source of treatment—they're also a source of information. Feel free to ask them any questions about aHUS.

Your loved ones

aHUS can take a lot out of you, so it's important to lean on friends and family for support. Be sure to let them know what aHUS is and how you're feeling day to day. That way they'll know when they need to reach out to lend a helping hand.

Your aHUS community

aHUS is a rare disease, but that makes those who have it feel that much closer. Connect with people with aHUS to help you learn about, cope with, and confront your disease.

There are a number of online resources you can use to find support (see page 21).

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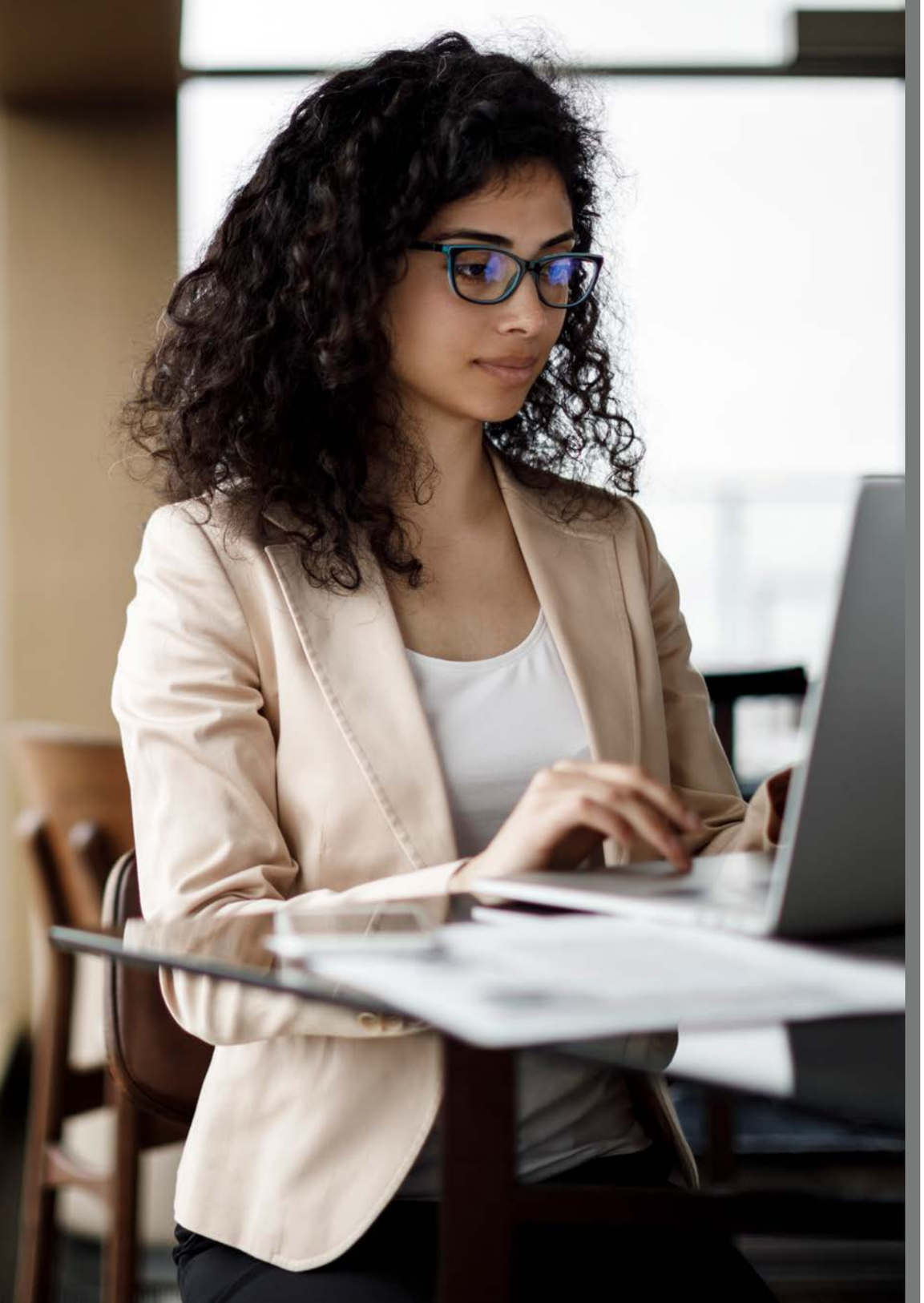

Helpful resources

Canadian Organization for Rare Disorders

Canadian Organization for Rare Disorders

Stay up to date with the latest findings on aHUS and other rare disorders.

raredisorders.ca

atypical Hemolytic Uremic Syndrome

Atypical Hemolytic Uremic Syndrome (aHUS) Canada

Get connected with other people living with aHUS and learn about services and organizations that can help support you and your family.

ahuscanada.org

ONESOURCE®
Personalized Patient Support from Alexion

ONESOURCE™

ONESOURCE™ Nurse Case Managers can help answer questions about aHUS and provide personal support for people living withaHUS and their caregivers. Additionally, Nurse Case Managers can help with the process and paperwork of getting started on treatment with SOLIRIS®.

1.888.765.4747

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aHUS is a SERIOUS condition.
aHUS is a disease that happens in the blood but can impact your entire body. And, even though it's always happening, your body may not always show signs or symptoms.

You CAN do something about it.
Even if you can't see or feel it, it's important to manage your aHUS. With the right tools and support, you can take control. Start by speaking with your doctor to find a treatment plan that's right for you.

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