For adult patients and caregivers

LEARNING TO LIVE WITH ATYPICAL-HUS



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aHUS SOURCE

ALEXION®
AstraZeneca Rare Disease

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WHAT IS **ATYPICAL-HUS?**

When the immune system spins out of control

Atypical hemolytic uremic syndrome (atypical-HUS) is a rare disease that happens when a part of the immune system called the complement system begins to overreact. This can be due to a gene mutation or can be triggered by another cause, such as medication, illness, or childbirth.

When the body can't control complement activity, complement attacks the body's own healthy blood vessel walls instead of the bacteria, viruses, and other foreign particles it usually attacks.

As complement keeps attacking blood vessel walls, blood clots form in the body's smallest blood vessels, reducing blood flow to various organs. At the same time, blood vessels become inflamed. This damage, inflammation, and clotting is called thrombotic microangiopathy, or **TMA**.

Left unmanaged, TMA can begin to damage important organs like the kidneys, heart, and brain. Damage from TMA can occur suddenly or gradually and can be life-threatening.



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ATYPICAL-HUS IS RARE– APPROXIMATELY **3** TO **9** **PEOPLE** OUT OF EVERY **1 MILLION** HAVE IT.

It occurs in similar rates in boys and girls, but far more women have it than men.

Historically, atypical-HUS was considered to be a "children's disease," but it's now known that atypical-HUS is more common in adults.

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WHAT ARE SOME **SIGNS** AND **SYMPTOMS** OF ATYPICAL-HUS?

Symptoms you may experience

Many of the symptoms of atypical-HUS are also signs and symptoms of TMA, the disorder that occurs when your body can no longer control complement activity. These signs and symptoms are common to many conditions. This can make atypical-HUS difficult to diagnose. Additionally, it's likely that no two people will share the exact same journey with this disease—you might experience only a few of these symptoms or have several of them.

ATYPICAL-HUS CAN DAMAGE SOME OF THE BODY'S MAJOR ORGANS AND SYSTEMS[a]



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Brain and Nervous System

- People with atypical-HUS may experience several serious symptoms associated with the brain and nervous system that include confusion, swelling of the brain, seizure, and stroke.

Eyes

- Your vision may become blurry or painful. The blood vessels of your retina may get blockages and you may even develop blood spots in your eyes—a sign of bleeding blood vessels.

Lungs

- Atypical-HUS can cause bleeding and fluid in the lungs or make it hard to breathe.

Heart

- People with atypical-HUS may experience symptoms associated with the heart. These symptoms can include blood clots forming in arteries, hardening and narrowing of veins, high blood pressure, heart disease, and heart attack.

Kidneys

- Kidney disease and kidney failure may occur in people with atypical-HUS.
- Testing for high levels of creatinine in the blood and high levels of protein in the urine can help show us that kidneys may not be functioning properly.
- eGFR is another test that can help tell how quickly a kidney is working. In patients with atypical-HUS, eGFR levels are often decreased, indicating that a kidney is not working as well as it should.
- When kidneys begin to fail, patients will often be placed on dialysis, a medical procedure that helps clean the blood. Sometimes a kidney transplant may be needed.

Digestive System

- There are several symptoms of atypical-HUS that affect the digestive system, also known as the gastrointestinal system. These include nausea and vomiting, diarrhea, and belly pains. Some people may experience swelling of different organs of the digestive system, including the stomach, intestines, and pancreas.

[a]These symptoms are not inclusive of all symptoms that may be associated with atypical-HUS.

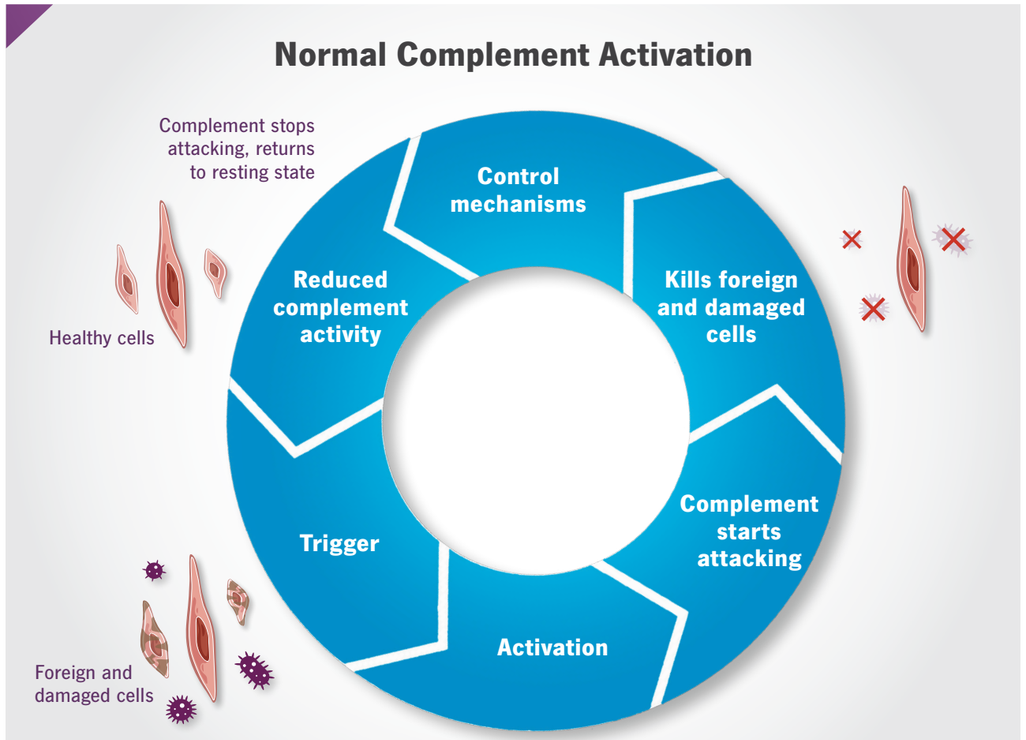
WHAT IS THE **COMPLEMENT SYSTEM?**

An important component of the immune system

The immune system constantly surveys the body for bacteria, viruses, and anything else that doesn't belong. The complement system helps to get rid of foreign and unwanted substances.

How complement normally works...

In normal situations, complement is a good thing. The immune system triggers complement when it needs help destroying foreign matter.



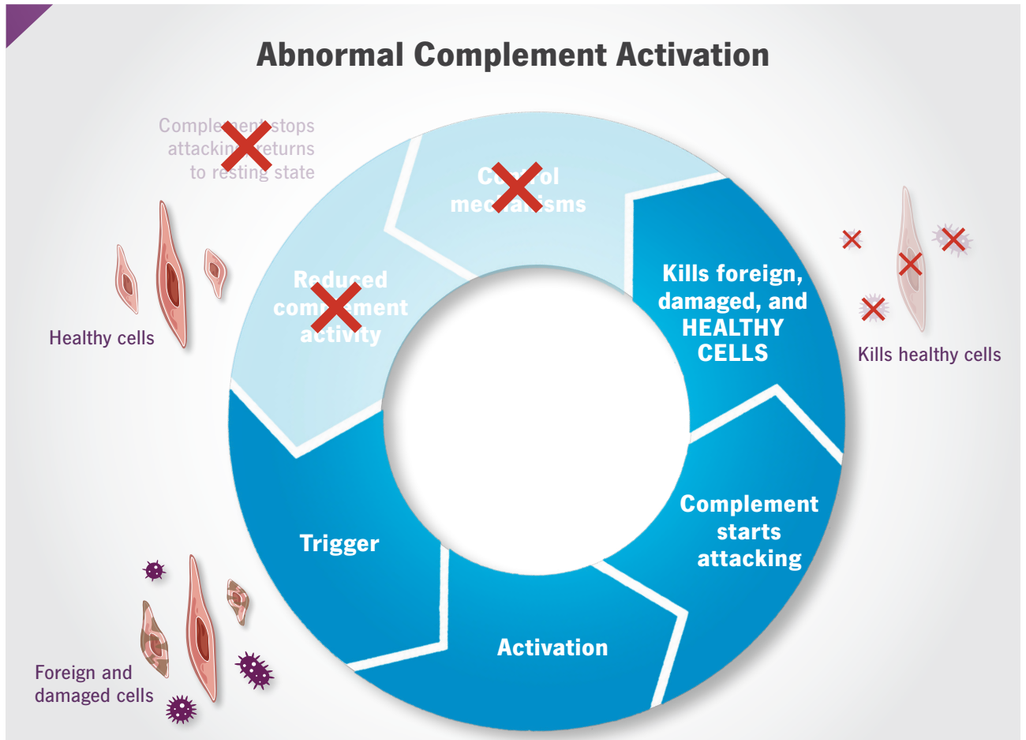
After damaged cells or foreign infections like bacteria have been removed, the body returns the complement cascade to a "resting state." This helps protect normal cells from damage.

The body also has ways to reduce and control complement activity when it is not needed.

THE **COMPLEMENT SYSTEM** IN ATYPICAL-HUS

...and why complement in atypical-HUS does not act normally

The problem in atypical-HUS is that complement becomes overactive—the body can't keep it under control. Once complement is triggered, it kicks into overdrive and doesn't turn off. It just keeps going—kind of like a hamster running on a wheel that never stops. Eventually, complement causes disease by attacking the body's own healthy cells and causing TMA.



In atypical-HUS, the body cannot reduce the activity of complement, leading to damage to the body's normal healthy cells.

In atypical-HUS, almost anything the immune system reacts to—stress, illness, surgery, infection, medication, even childbirth—could kick complement into overdrive and cause TMA.

" Know that the knowledge about our disease improves all the time."

- Magnus

Computer programmer and father, living with atypical-HUS in Sweden



While atypical-HUS may feel overwhelming, **there are ways that your doctor can help you manage it.**

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HOW IS ATYPICAL-HUS **DIAGNOSED?**

There is no single test to diagnose atypical-HUS

Atypical-HUS is a rare disease. It can closely resemble other diseases that can cause TMA, like thrombocytopenic purpura (TTP, a condition in which clots form in blood vessels) and STEC-HUS (HUS that's caused by a toxin instead of an immune system malfunction). This can make it hard for doctors to diagnose atypical-HUS.

Doctors have to run several tests to exclude the possibility of these other disorders in order to confidently determine if someone has atypical-HUS.

Here are some tests they may run and the results to look for:

- **Low platelet counts**

  Platelets play a strong role in forming blood clots. Depending on how low your platelet levels drop, your body may have trouble forming blood clots after injury. Early signs include bleeding gums and small purple, red, and brown dots—or bruises—under the skin.

- **High lactate dehydrogenase (LDH) levels**

  High LDH levels are a sign of tissue damage, including hemolysis, which occurs when red blood cells are torn apart and become schistocytes during TMA.

- **Low eGFR**

  Doctors use eGFR to gauge how well your kidneys are working. A low eGFR can mean you have some type of kidney damage.

- **High creatinine**

  Creatinine is a protein that, when at higher-than-normal levels, can be a sign of kidney damage.

Although these are the tests commonly used to help diagnose atypical-HUS, doctors may also run other tests to confirm a diagnosis. Please see **Helpful Terms** on page 15 for more information.



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The Good News: atypical-HUS can be managed

You should work closely with your doctor to build a management plan that works for you.

Remember to always tell your doctor about any potential symptoms that you experience. These symptoms may include feeling a little confused or weak, having trouble breathing, or being really tired. There are many possible symptoms associated with atypical-HUS, and your healthcare team is here to help.

Here are a few tips when preparing to speak with your healthcare team

- Have a journal so you can track and discuss your symptoms
- Write down questions and topics for discussion
- Ask if you can record the appointment
- Don't be afraid to ask your doctor to clarify anything you don't understand
- Keep copies of all your test results



Caring for mind, body, and spirit

Atypical-HUS can seem overwhelming, but there are ways you can help yourself through your journey. Always talk with your doctor before changing diet, physical activity, and/or daily lifestyle routines.

- **Try to slow down and take one day at a time. Pacing yourself can help preserve your energy.**
  - Atypical-HUS can sometimes be draining. Learning how to pace yourself may allow you to still enjoy many of the same activities as before. Consider keeping a planner to track your activities. This may help you pinpoint when you may be pushing yourself too hard. That way, you can plan accordingly and get the most out of each day.
- **Don't be afraid to speak up. Mental wellness is important.**
  - It is okay to ask for help.
  - If you have any concerns, never feel ashamed about discussing them with your doctor. They may have suggestions to help you cope with your atypical-HUS journey.
- **Develop a support system.**
  - Confide in and rely on friends and family.
  - Join a support group. Reaching out to other people with atypical-HUS can be helpful.
  - Rely on your healthcare team.
  - Alexion's OneSource™ patient services program can also provide support to eligible patients.

Here are a few examples of resources for taking care of mind-body wellness. These examples of general mind-body wellness resources listed below do not treat aHUS and are not a substitute for individualized medical care provided by a qualified physician. These sites are not affiliated with or controlled by Alexion Pharmaceuticals, Inc., and Alexion does not influence and is not responsible for their content.

mindbodygreen.com

- A website that works to help inspire you to live your best life by giving you tips on mental, physical, spiritual, emotional, and environmental wellness needs.

selfcareday.com

- A website by the Crisis Text Line, SelfCareDay.com offers readers a variety of tools for self-care so you can tailor solutions to fit your own individual needs.

zenhabits.net

- This website focuses on helping users become more mindful. Resources include workshops, books, and training.

themindbodyspiritnetwork.com

- If holistic health and alternative wellness interest you, you'll find a wide variety of resources to support you in your health journey, ranging from a life coach to online communities and summits.

calm.com

- A meditation website and app, Calm.com offers a free trial. As a new user, you'll answer a series of questions about the causes of your stress, your health goals, and your experience in meditation to customize a program that gets you started.

YOU ARE **NOT ALONE** IN THIS

Having atypical-HUS can be scary, but there's plenty that can be done to feel more in control of your life. Best of all, atypical-HUS is not something you have to go through alone. There is hope. There are people you can turn to for support.

- Sign up for more information at aHUSSource.com.
- Get access to 1:1 education and community events by connecting with your OneSource Patient Education Manager at AlexionOneSource.com.
- Talk with your doctors, nurses, or other members of the office staff who can answer your questions and connect you with other professionals who can help you.
- Reach out to the community of other caregivers and patients dealing with atypical-HUS.

"It may seem like your world is turned upside down, but it does get better."

- April

Penguin and hockey lover,
living with atypical-HUS in Ohio, USA

ONESOURCE®
Personalized Patient Support from Alexion

OneSource is a complimentary, personalized support program offered by Alexion. OneSource is staffed by Alexion Patient Navigators, Patient Liaisons, and Patient Education Managers, all of whom have extensive knowledge of atypical-HUS and can assist you every step of the way. They are ready to provide the support and resources you need—wherever you are in your atypical-HUS journey.

Learn more about atypical-HUS by attending aH**US Together** Alexion events at alexionaHUSevents.com, where you can learn from expert physicians and hear stories from other people living with atypical-HUS.

OneSource can provide helpful resources and tools to get you started on your atypical-HUS journey.

Connect with OneSource so we can partner you with a Patient Education Manager to begin helping you today. Though atypical-HUS is rare, it's not rare to us. To get started, visit AlexionOneSource.com or call 1.888.765.4747.

SUPPORT GROUPS

Atypical-HUS can be a lot to deal with, and sometimes you need a shoulder to lean on. These support groups can help when you're working through your challenges:

aHUS SOURCE

aHUS Source

Provides patients and caregivers information on atypical-HUS, along with a guide to help you and your doctor work together to manage atypical-HUS. aHUSSource.com

NORD® National Organization for Rare Disorders

National Organization for Rare Disorders (NORD)

Dedicated to helping people with rare, or "orphan," diseases. This organization helps patients access assistance programs, hosts meetings for patients with atypical-HUS and their families to help them connect to others, and provides information on rare diseases by sharing patients' personal stories. rarediseases.org

Global Genes™ A RARE PROJECT™

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 atypical-HUS and their families across the country. globalgenes.org

aHUS Source

The purpose of the aHUS Source Facebook page is to raise awareness of atypical-HUS and build an active community of patients, caregivers, and advocates to learn together and share their experiences living with the disease with each other. facebook.com/aHUSSource

aHUS FOUNDATION® SHAPING OUR PATH FORWARD

aHUS 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 atypical-HUS. The overall goal is to gather people together to improve the lives of patients and families dealing with atypical-HUS. aHUS.org

NATIONAL KIDNEY FOUNDATION®

National Kidney Foundation

Dedicated to preventing kidney disease, improving the health and well-being of individuals and families affected by kidney disease, and increasing the availability of kidneys for transplantation. kidney.org

American Kidney Fund® FIGHTING ON ALL FRONTS

American Kidney Fund

Fighting kidney disease and helping people live healthier lives–with an unmatched scope of programs that support people wherever they are in their fight against kidney disease–from prevention through post-transplant living. kidneyfund.org

aHUS Alliance www.aHUSalliance Action.org

aHUS Global Alliance

Through the collaboration of its affiliates, the aHUS Global Alliance will promote global awareness of aHUS, work with international aHUS researchers, and, by supporting newly emerging national aHUS patient groups, bring relief and support to those affected by aHUS to save, and improve the quality of, more lives. aHUSAllianceaction.org

CAREGIVERS NEED **SUPPORT**, TOO

Caring for someone with atypical-HUS includes getting the support you need

Dealing with atypical-HUS can be a scary and challenging journey for both patients and caregivers. It may bring changes to your daily life and can feel overwhelming at times.

It is important to know that you don't have to do this alone. It is okay to ask for help, and there are people you can turn to for support.

Build a partnership with your healthcare team

The doctors, nurses, and other members of the office staff are there for both you and your loved one. They can not only help answer questions about atypical-HUS, but also help you be an advocate for your loved one's health.

Join a support group

The same support groups that are helpful for patients can be a resource for you, too. Even though everyone's journey with atypical-HUS may be different, reaching out and connecting to other caregivers and patients can provide comfort, perspective, and an important supportive community. Other atypical-HUS caregivers are on journeys that may be similar to yours.

Utilize OneSource

OneSource, the program that connects you with an Alexion Patient Education Manager, can offer you the same support and assistance it offers to patients.



OneSource can provide helpful resources about atypical-HUS. Contact OneSource at 1.888.765.4747.

HELPFUL **TERMS**

- **Blood transfusion:** describes the process of replacing blood lost by surgery or trauma.
- **Complement system, or complement:** a part of the immune system that helps the body destroy certain viruses, bacteria, and damaged cells. Complement is the system that becomes overactive in people with atypical-HUS, where it can start attacking healthy blood vessel walls.
- **Creatinine:** a natural waste product that is normally filtered by the kidneys and removed by urination. High creatinine levels in the body can be a sign of kidney disease.
- **Dialysis:** a medical treatment that mimics kidney function by filtering the blood and removing toxins, fluid, and waste.
- **Estimated glomerular filtration rate (eGFR):** a test that describes how quickly kidneys are working.
- **Lactate dehydrogenase (LDH):** an enzyme that helps the blood break down sugar to create energy. Damaged and ruptured cells release LDH, too, and LDH runs high in atypical-HUS.
- **Platelet:** a disc-shaped cell fragment found in the blood that assists in forming blood clots.
- **Schistocytes:** broken pieces of red blood cells.
- **Shiga toxin–producing *E. coli* hemolytic uremic syndrome (STEC-HUS):** a rare disease and the most common cause of kidney failure in children. This condition occurs when Shiga toxin–producing *E. coli* triggers complement to attack the kidneys.
- **Thrombotic microangiopathy (TMA):** a group of medical disorders associated with damage to small blood vessels, blood clots, and reduced blood flow, which results in injury to organs.
- **Thrombotic thrombocytopenic purpura (TTP):** a rare blood condition that causes blood clots in tiny blood vessels that can block healthy blood flow to important organs in the body like the heart, brain, and kidneys.

" You don't need to be strong. You don't need to be perfect. Just remember, you are not alone on your journey. "

- Middle school student with dreams of becoming a doctor
  Diagnosed with atypical-HUS 3 years ago



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YOU ARE NOT ALONE ON YOUR JOURNEY.

ONESOURCE CAN PROVIDE HELPFUL RESOURCES ABOUT ATYPICAL-HUS. CONTACT ONESOURCE AT 1.888.765.4747 OR SIGN UP ONLINE AT **ALEXIONONESOURCE.COM**.

aHUS SOURCE

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