For pediatric patients and caregivers

LIVING WITH ATYPICAL-HUS
YOU DON'T HAVE TO FACE IT ALONE


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aHUS SOURCE

ALEXION®
AstraZeneca Rare Disease

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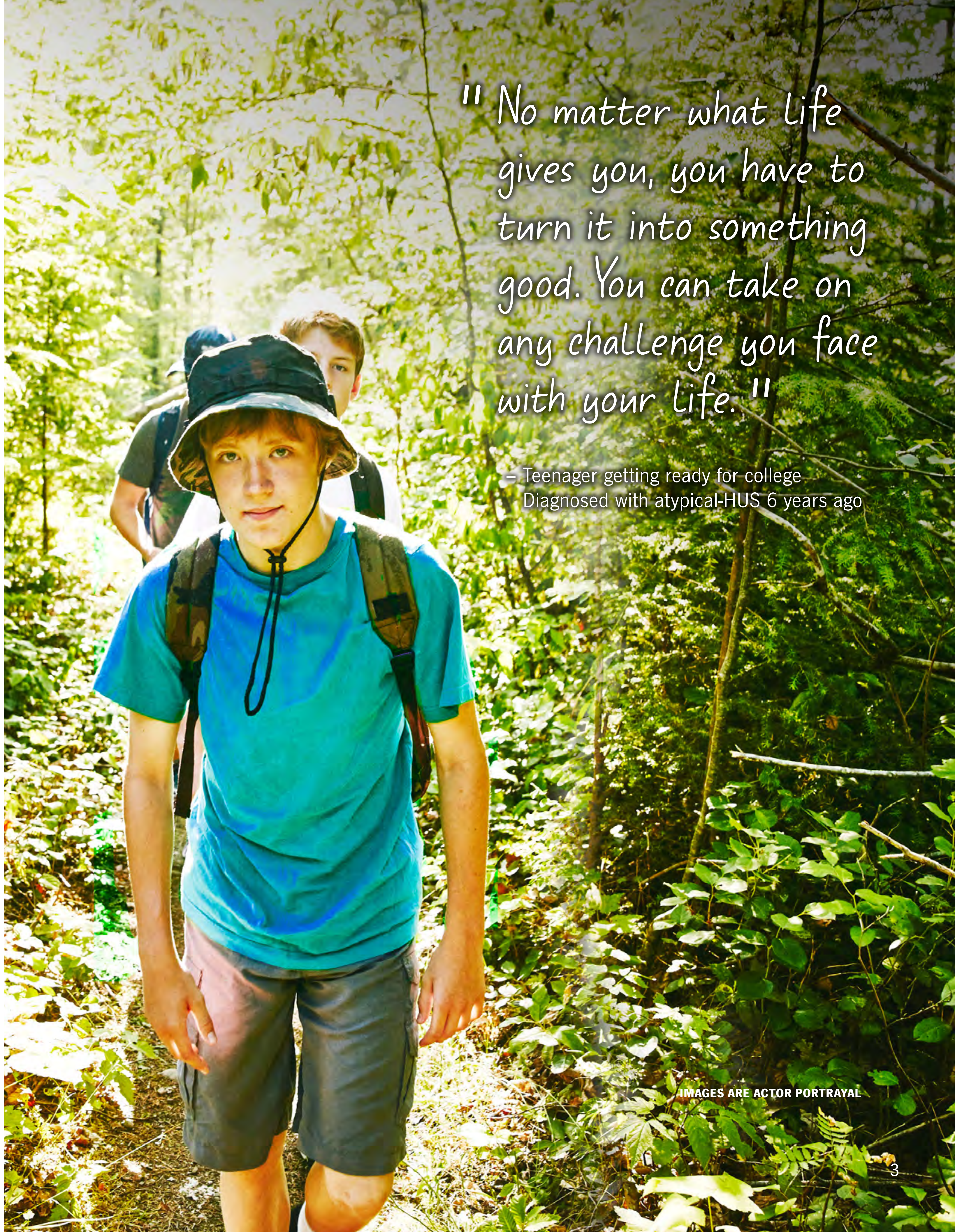

"No matter what life gives you, you have to turn it into something good. You can take on any challenge you face with your life."

– Teenager getting ready for college
Diagnosed with atypical-HUS 6 years ago

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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 in which the body's immune system doesn't function as it should. The immune system helps keep us safe from getting sick. With atypical-HUS, the complement system—a part of the immune system—can't be controlled and kicks into overdrive. As a result, complement attacks your body's blood vessel walls instead of the viruses and other nasty bugs that it normally attacks.

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

If this TMA isn't treated, it can damage important organs like the kidneys, heart, and brain. The damage can happen suddenly or gradually.

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

In children, atypical-HUS occurs at similar rates in boys and girls.

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

How a child may feel

Atypical-HUS can be tricky for doctors to find, so it's important to know what to look for. Many of the symptoms of atypical-HUS are also the signs and symptoms of TMA, and TMA can occur with other diseases. This can make atypical-HUS difficult to diagnose. No two people will share the exact same journey with this disease—someone might experience only a few of these symptoms or have several of them.

ATYPICAL-HUS CAN HARM IMPORTANT ORGANS IN THE BODY[a]

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

- Atypical-HUS can cause painful and blurry vision.

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.

COMPLEMENT IS YOUR FRIEND

Children and puppies are often inseparable duos, exploring the world together from one adventure to the next. They are best friends who care for each other deeply.

However, even the sweetest puppies can misbehave from time to time. Maybe they have an accident in the house or decide that eating some homework for lunch sounds like a good idea.

Usually, with a firm command, a pat on the head, or maybe even a treat, the puppy will stop misbehaving. Soon enough, they're back to playing fun games with their best friend.

Just like the puppy, the complement system is the best friend to the rest of the immune system. Together they work to keep our bodies safe from things such as viruses and bacteria.

WHEN **COMPLEMENT MISBEHAVES**

Normally, our bodies control the behavior of complement so it doesn't begin misbehaving while it works to help protect us from invading viruses and bacteria.

However, with atypical-HUS, our bodies can no longer control complement activity, and the complement system can start to misbehave.

- In this case, once complement is triggered, it can't stop. It begins to become overactive. Complement will eventually attack and damage your body's blood vessel walls, causing TMA.
- Many things—such as stress, illness, surgery, and infection—can cause complement to go into overdrive, triggering TMA and leading to the signs and symptoms of atypical-HUS.
- Atypical-HUS is a lifelong disease, with an ongoing and unpredictable risk of complement being triggered into damaging your blood vessels and organs.

Just like you might use a firm command or a treat to help calm down a misbehaving puppy, your doctors have ways to help you manage your atypical-HUS.

Complement protein 5, or C5, is an important part of the complement system. C5 helps the complement system get ready to destroy dangerous or damaged cells. Once the threat is gone, the body normally helps C5 and the rest of the complement system get back under control.

In atypical-HUS, the body can't get C5 and complement under control like it normally would, and uncontrolled C5 activity begins to cause damage to healthy cells.

> "New things you learn at different parts in the journey will give you momentum to take the next step."
>
> – Proud mother of a child with atypical-HUS
> Diagnosed 7 years ago

While atypical-HUS may feel overwhelming, **working with doctors can help manage symptoms associated with this disease.**

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HOW DOES A DOCTOR KNOW WHEN SOMEONE HAS ATYPICAL-HUS?

There is no single test to diagnose atypical-HUS

Atypical-HUS is a rare disease that acts a lot like other diseases that also cause TMA. Onset of atypical-HUS can be sudden or gradual, can be life-threatening, and can occur at any age. This makes it harder for doctors to diagnose atypical-HUS.

In fact, doctors have to run several tests to rule out other conditions before they know for sure that someone has atypical-HUS.

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

- **Low platelet counts**

  Platelets play an important role in forming blood clots that are necessary in healing. Depending on how low platelet levels drop, the body may have trouble forming blood clots after an injury.

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

  LDH is a special kind of protein, called an enzyme, that speeds up reactions our bodies carry out. High LDH levels are a sign of tissue damage including hemolysis, which occurs when red blood cells are damaged and become schistocytes.

- **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 higher than normal can be a sign of kidney damage.

These tests are commonly used. Your doctor will probably perform lots of other tests to confirm an atypical-HUS diagnosis, including specific tests to rule out other diseases. Please see **Helpful Terms** on page 15 for more information.

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HOW IS ATYPICAL-HUS **MANAGED?**

The Good News: atypical-HUS can be managed

Parents and caregivers should work closely with their child's healthcare team to build a management plan that works for them

Remember to always tell your doctor about any potential symptoms that your child experiences. 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
- Ask if the doctor can explain complex topics in a "kid-friendly" way
- Encourage children to ask questions if they don't understand something

Remember to speak with your doctor about your child's symptoms and how to manage atypical-HUS.

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FINDING WAYS FOR A KID TO BE A KID

Caring for mind, body, and spirit

Living with atypical-HUS can be hard, but there are ways to help a child through this journey. Always talk with your doctor before changing diet, physical activity, and/or daily lifestyle routines.

- **Everyone experiences atypical-HUS differently**
  - Consider using a planner to track daily activities, which can help pinpoint when things are getting overwhelming.
  - Understanding how this disease may affect energy levels can be very helpful. Soon you may be able to enjoy many of life's little joys again.
- **Speak up and ask for help if needed**
  - Mental wellness is important.
  - No matter the concern, no one should feel ashamed about discussing things with a doctor. They may have suggestions on how to cope with atypical-HUS.
- **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.
  - 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.

headspace.com

- One of the first meditation apps available, Headspace provides opportunities for mindfulness techniques for both adults and children.

mindbodygreen.com

- This website from the Crisis Text Line offers readers a variety of tools to help with self-care in daily life, with a focus on confidence, stress, and wellness.

calm.com

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

my.life.com

- This subscription site works by providing mindfulness activities and techniques for parents and children, based on their specific emotional needs.

zenhabits.net

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

YOU ARE **NOT ALONE** IN THIS

Having atypical-HUS can be scary, but it's not something you and your loved ones 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.

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.

> "OneSource became a second family. They were always there to respond to our questions."
>
> **- Susan**
> Parent of a child with atypical-HUS

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

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

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

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

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

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 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 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

PARENTS AND **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 children, parents, and caregivers. It may bring changes to your daily life. It can be stressful or isolating 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.

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.

> "Take a deep breath, ask for help, find a support system, and focus on one good thing every single day. It's a continuous process."
>
> – Caregiver of a young girl diagnosed with atypical-HUS 4 years ago

Join a support group

The same support groups that are helpful for patients can also be helpful for you. 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.

There are many potential resources for caregivers, including Angel Aid (www.angelaidcares.org), a support group for caregivers of children with rare diseases.

Build a partnership with your healthcare team

Your doctors, nurses, and other members of the office staff are there for you and your loved ones. They can help answer questions about atypical-HUS, provide resources, and help you be an additional advocate for your loved one's health.

OneSource can provide a free atypical-HUS starter kit with 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.
- **Blood clot:** a clump that occurs when blood hardens from liquid to solid. Depending on the size of the clot and the size of the blood vessel it is passing through, the clump can act like a plug that blocks normal blood flow. This can cause organs to become damaged.
- **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.
- **Platelets:** 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**.

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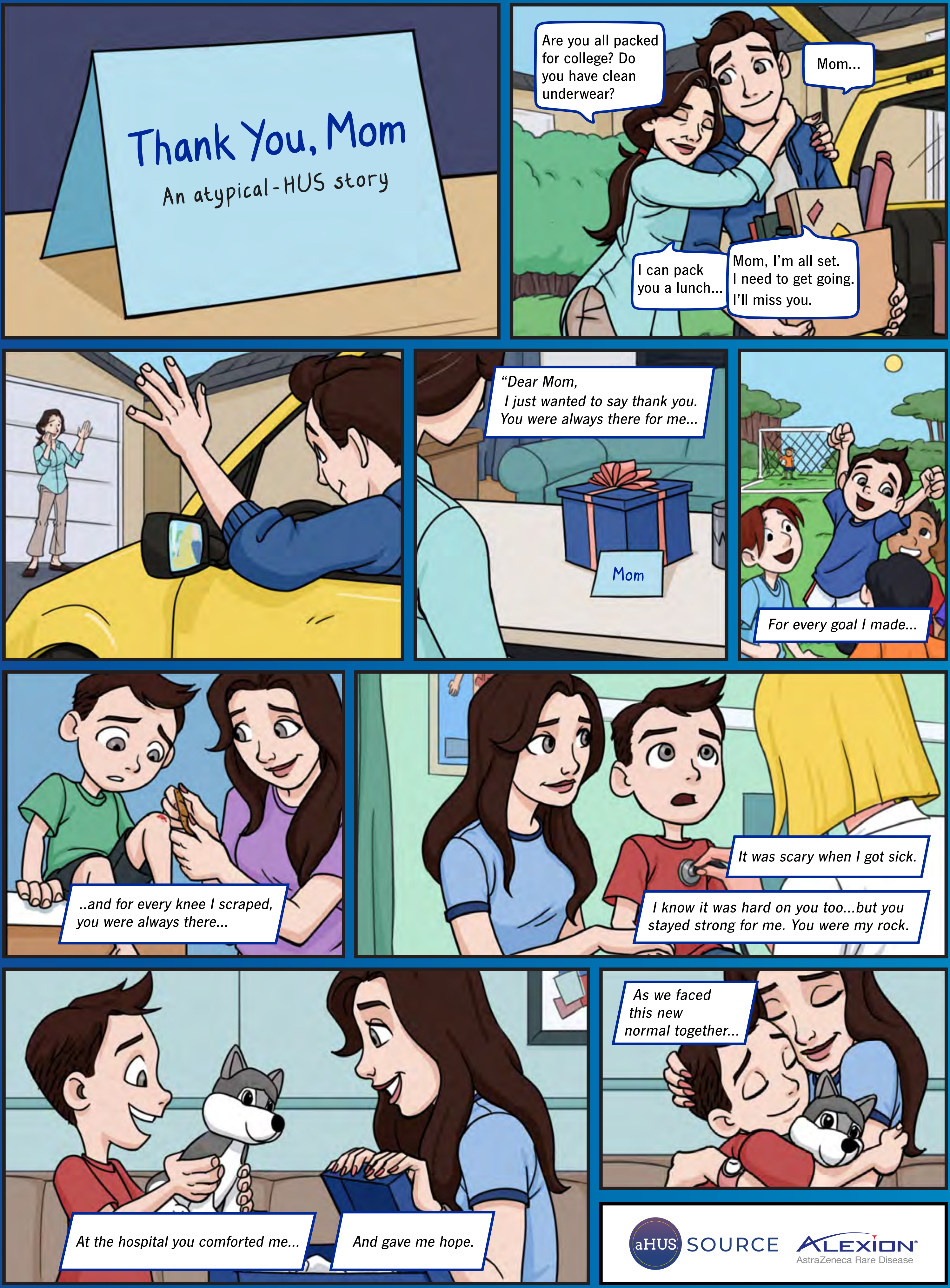

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