A CAREGIVER'S JOURNEY

Learning about atypical hemolytic uremic syndrome (aHUS)

Begin your path to effectiv advocacy and support

Caring for someone who is diagnosed with aHUS can be overwhelming. You may have many questions: What is aHUS? How do you get it? Who can I turn to for help? This brochure will help you learn more about the diagnosis of aHUS and will guide and support you as a caretaker.

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 terms used when aHUS is discussed may be unfamiliar to you. They have been called out in boldor green text, and their de nitions can be found toward the back of this brochure.

“Caring for someone with
and finding the right suppor

Notes

Some caregivers find it helpful to take notes when talking to health care providers, nurse Case Managers, and others. Taking notes may help you keep track of these conversations.

aHUS can be a very scary journey. Understanding th
rt has made it easier on both of us.”

– Caregiver to someone with aHUS

A CAREGIVER'S JOURNEY

Learning about aHUS

The journey begins: What is aHUS?

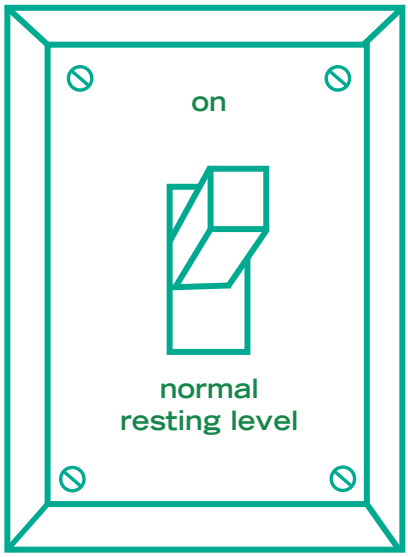
aHUS is a disorder caused by a genetic change (such as a mutation) in the complement system, which is part of the immune system.[1,2] The complement system is always "on" at a low level and ready to increase activity to protect the body from infection.[3,4] Everyday occurrences, including infections, allergies, and even pregnancy, can increase complement activation.[2,5–10]

Normally, the body is able to recognize when complement levels should return to normal. In people with aHUS, the body has a problem regulating complement, which causes it to be uncontrolled due to the changes in the complement regulatory genes.[1,3,6]

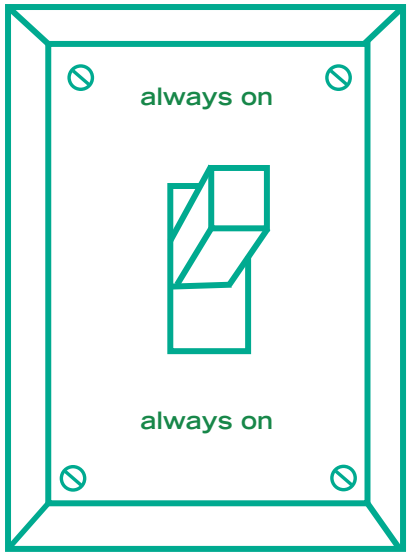
How long will someone have aHUS?

aHUS is a genetic disease, meaning that it is caused by a change, or mutation, in a person's DNA.[1–3] This means that he/she will always have aHUS.[1,2] These mutations make it difficult for the body to regulate, or "turn off," the complement system.[2,11,12]

Complement activity can be increased by everyday occurrences such as a bacterial or viral infection, pregnancy, allergy, and other trauma.[2,5-9]



versus



In people without aHUS:

The complement system is turned on by everyday occurrences.[3] After the need is gone, the body regulates the complement system by turning it to normal levels.[1,3,6]

In people with aHUS:

The body cannot regulate the complement system.[1,13] It remains turned on, or "activated."[1,3] This uncontrolled complement activation can cause damage to many parts of the body.[14-18]

What you may encounter on your journey: Signs and symptoms of aHUS

When someone has aHUS, increased complement activity can lead to damaged cells along blood vessel walls and platelets becoming overactive.[5,17,19] This can result in blood clots that form in the small vessels that go to the kidneys, heart, brain, digestive tract, and other organs causing harm or impairment.[5,17,19]

A person with aHUS may have already experienced some of the signs and symptoms listed below. If you think he/she is experiencing any of these symptoms, contact a doctor right away. Understanding aHUS and recognizing its signs and symptoms are important steps in managing the effects of the disease.

KIDNEYS

>50% of all patients with aHUS have kidney complications

- Dialysis (end-stage renal disease)[14]

BRAIN

48% of patients with aHUS have neurologic symptoms[15]

- Confusion[20]
- Stroke[20]
- Seizure[15]

“I was under the impression that aHUS is a blood disease that involves only your kidneys... I didn’t realize the brain and heart can also be involved.”

– Patient diagnosed with aHUS

HEART

43% of patients with aHUS have cardiovascular symptoms[15]

- Heart attack[18,21]
- High blood pressure/hypertension[22]
- Blood clots[17]

DIGESTIVE TRACT

37% of patients with aHUS experience gastrointestinal symptoms[17]

- Abdominal pain[23]
- Diarrhea[10,24]
- Colitis[8]
- Nausea/vomiting[25]

aHUS is a chronic disease

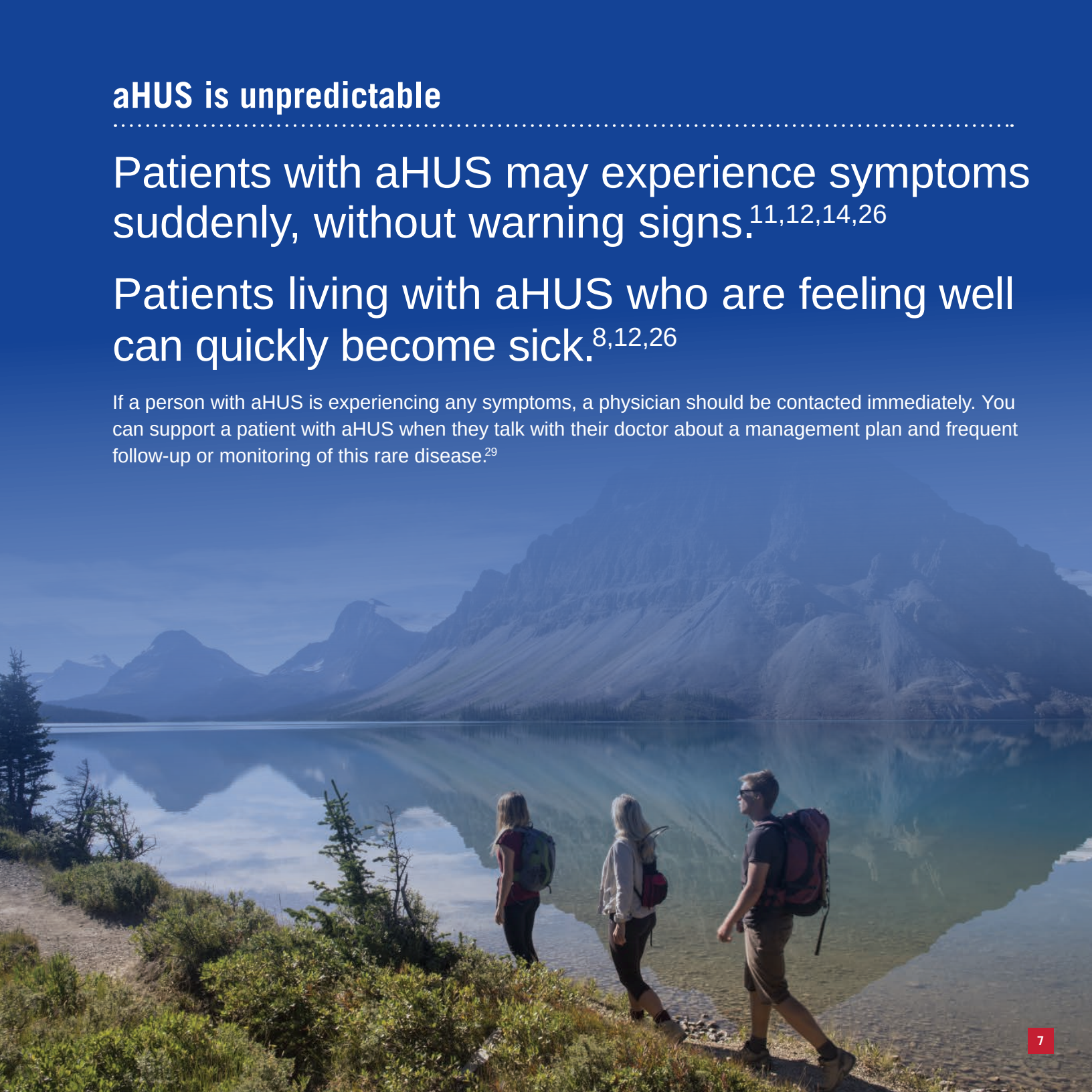
Every person with aHUS is different. Some may go for long periods without symptoms, but others may feel sick frequently.[1,27]

aHUS is unpredictable

Patients with aHUS may experience symptoms suddenly, without warning signs.[11,12,14,26]

Patients living with aHUS who are feeling well can quickly become sick.[8,12,26]

If a person with aHUS is experiencing any symptoms, a physician should be contacted immediately. You can support a patient with aHUS when they talk with their doctor about a management plan and frequent follow-up or monitoring of this rare disease.[29]



“Support is available at the time of diagnos
and will continue as you manage aHUS.”

– Patient diagnosed with aHUS

Getting oriented: Working with a health care team

Here are a few questions you may want to consider asking the health care team:

How is aHUS managed?

What are the management options?

What kind of home care is needed, if any?

How often should doctor’s visits be scheduled?

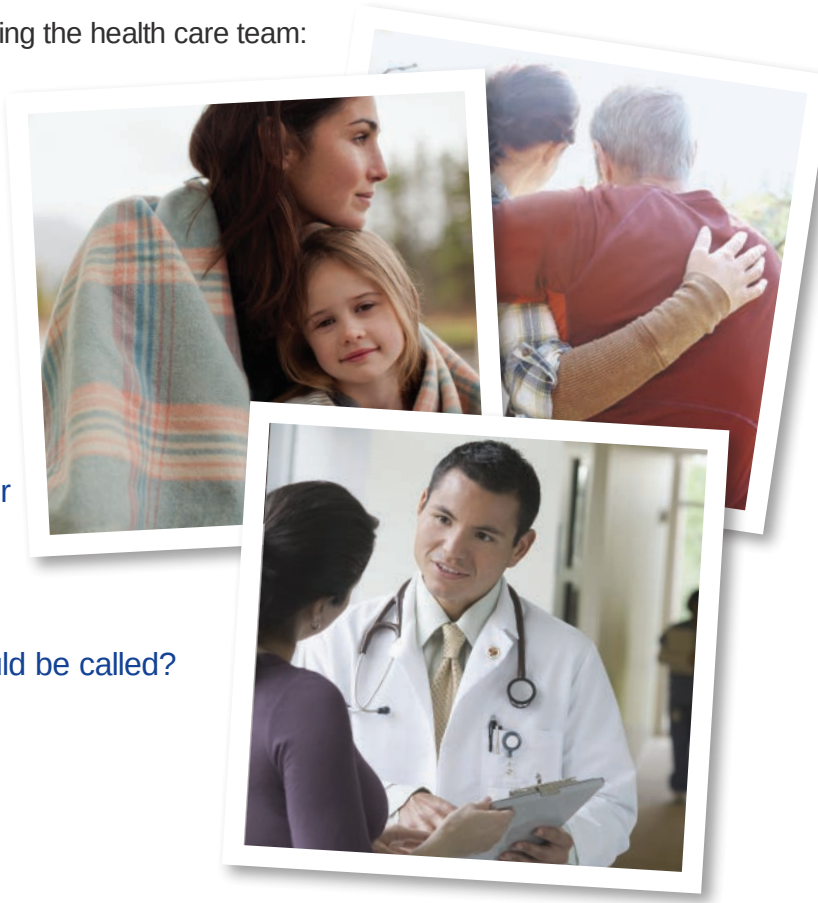
What tests will be done to evaluate this disease?

- What will the results tell me?

What are important signs and symptoms to monitor at home?

What should we do if symptoms occur?

If a person with aHUS is not feeling well, who should be called?



Staying on track:
Caring for someone with aHUS and getting the support you need

Caring for someone with aHUS may bring changes to your daily life, including some challenges. This can be an emotional journey, and it is important to know that there are people you can turn to for support.

> “It does get better. It’s scary at first, but it does get better.”
>
> - Patient diagnosed with aHUS

The first step in caring for someone with aHUS will likely be to start to build a partnership with the health care team. This team may include doctors, nurses, and other members of the office staff. Team members can answer questions that you may have about aHUS. Along with the health care team, a variety of resources are available to guide you and provide support for you as a caregiver.

You may find it helpful to talk to other caregivers and patients dealing with aHUS. Although the exact path is different for everyone, other aHUS caregivers are on journeys that may be similar to yours.

OneSource™ is a program that will connect you with Alexion Nurse Case Managers who are experienced in working with patients with aHUS and their caregivers. Keep reading to nd out more about this valuable program.

ONESOURCE™

Personalized Patient Support from Alexion

Living with aHUS can be a challenge:
Although aHUS is rare, support is not hard to nd

OneSource is available to provide essential support, regardless of the management plan you choose.

- OneSource is a complimentary program offered by Alexion
- OneSource is staffed by Case Managers: registered nurses with disease knowledge and funding expertise
- OneSource can help patients facilitate the transition from hospital to home
- OneSource Case Managers are assigned geographically, so they are familiar with physicians, hospitals, community resources, and funding options unique to your area

OneSource Case Managers provide ongoing support in the following areas:

- Education: Collaborating with your healthcare team, to answer questions, and provide educational materials related to aHUS
- Coverage issues and funding options: Assisting patients and practices with insurance coverage questions and researching funding options
- Management support: Assisting with solutions for balancing all aspects of disease management when faced with major life challenges, such as college, relocation, marriage or divorce, retirement, or changes in insurance coverage

"This disease is very rare. But it's not rare to me."

– OneSource Nurse Case Manager

“If I needed information or had questions,
I would contact OneSource…
it is a support system, a way to make su
you don’t fall through the cracks.”

– Patient diagnosed with aHUS

Call OneSource at 1.888.765.4747 to speak one-on-one with a OneSource Case Manager. Case Managers can assist you with educational resources and information about funding options and assistance.

To learn more about OneSource,
visit www.ahussource.com/Patient/OneSource

Support groups: Find other people who are living with aHUS

Caring for someone with aHUS can feel overwhelming. Below are some resources that can provide additional guidance.

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 with a 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

aHUS FOUNDATION
SHAPING OUR PATH FORWARD

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

Personalized Patient Support from Alexion

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 knowledge of aHUS.

Website: www.ahussource.com/Patient/OneSource

Glossary

Basic metabolic panel (BMP) is a set of blood chemical tests (ie, Chem-7 or Chem-10) that provides key information regarding uid and electrolyte status, kidney function, blood sugar levels, and response to various medications. It is frequently employed as a screening tool during a physical exam.

Cardiovascular means involving the heart and/or blood vessels.

Colitis is swelling or in ammation in the large intestine, or colon.

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

Complete blood count (CBC) is a blood panel test that measures red blood cells, white blood cells, hemoglobin, and platelet count to provide a broad view of the cells in a patient's blood. This test can help a doctor diagnose abnormalities and evaluate cell production and breakdown.

Creatinine is a chemical waste molecule that is generated through muscle metabolism and is transported through the bloodstream to the kidneys. Creatinine has been found to be a fairly reliable indicator of kidney function; an elevated creatinine level in the blood can signify impaired kidney function or kidney disease.

End-stage renal disease (ESRD) occurs when the kidneys can no longer function to remove waste and excess water from the body. Patients with ESRD must undergo hemodialysis to lter their blood.

Endothelial cells form the inner lining of a blood vessel and provide an anticoagulant barrier between the vessel wall and the blood. These cells are involved in the immune response, blood clotting, in ammation, blood pressure, and organ repair.

Genetic disease is a disease caused by a genetic mutation.

Hematologist is a doctor who specializes in treating patients with diseases involving the blood.

Hemodialysis is a procedure used to lter blood when the kidneys are not functioning properly. Blood ows through a lter that removes waste and water, and then is returned to the body.

Hemoglobin is the protein molecule in red blood cells that carries oxygen from the lungs to body tissues and returns carbon dioxide from the tissues to the lungs.

Hemolytic anemia is a condition in which red blood cells are destroyed and removed from the bloodstream before their normal lifespan is over. When blood cells die, the body's bone marrow makes more blood cells to replace them. However, in hemolytic anemia, the bone marrow cannot make red blood cells fast enough to meet the body's needs.

Hypertension is another term for high blood pressure.

Lactate dehydrogenase (LDH) is an enzyme that is found in almost all of the body's cells and is released from cells into the blood when cells are damaged or destroyed. Thus, the blood level of LDH is a general indicator of tissue and cellular damage.

Mutation is a permanent change in the DNA sequence that makes up a gene. Genes code for proteins, so changes that occur in the DNA sequence can lead to proteins that do not function properly.

Nephrologist is a doctor who specializes in the kidneys and in kidney diseases.

Neurologic means involving the nervous system, including the brain, spinal cord, and nerves.

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

Proteinuria is a condition in which urine contains abnormal amounts of protein. As blood passes through healthy kidneys, they filter out waste products and leave in proteins and other things the body needs. However, proteins from the blood can leak into the urine when the filters of the kidney are damaged. Proteinuria is a sign of kidney disease.

Red blood cells (or erythrocytes) are the most common type of blood cell. They contain hemoglobin and are the body's principal means of delivering oxygen to body tissues via the circulatory system.

Seizure is caused by changes in the activity of nerve cells within the brain. Seizures can vary in intensity from barely recognizable events to loss of consciousness and convulsions.

Stroke is a medical emergency caused by lack of blood flow to the brain, causing death of brain cells.

Thrombosis is the formation of a blood clot inside a blood vessel. This leads to a blockage in the flow of blood through the blood vessels.

Thrombotic microangiopathy (TMA) is a disorder that results in clots and inflammation in various small blood vessels throughout the body, due to an endothelial injury.

Urinalysis is the physical, chemical, and microscopic examination of urine. It involves several tests that detect and measure various compounds that pass through the urine.

White blood cells (or leukocytes) are the cells of the immune system that are involved in defending the body against disease. White blood cells are produced in the bone marrow and are found throughout the blood and lymphatic system. The number of white blood cells in the blood is often an indicator of disease.

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