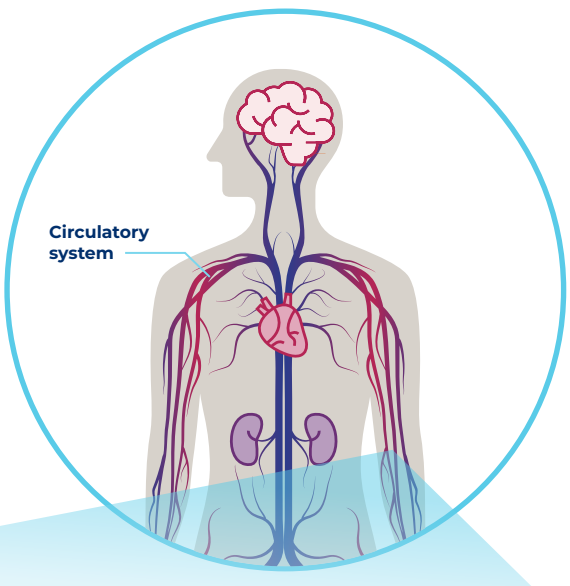
Atypical Haemolytic Uraemic Syndrome (aHUS)

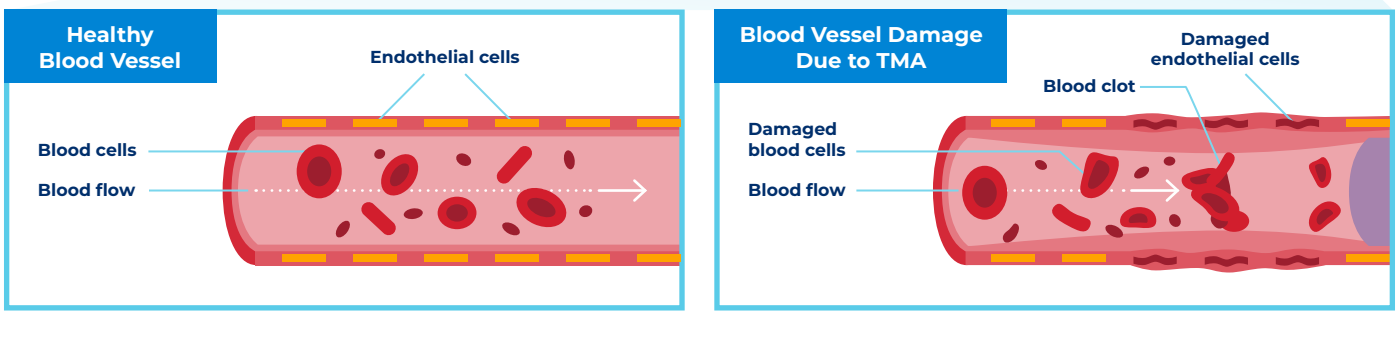
AstraZeneca Rare Disease

WHAT ARE TMAs?

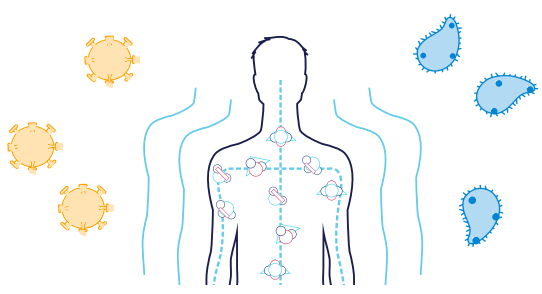
Thrombotic microangiopathies (TMAs) are a **group of severe and potentially life-threatening rare disorders that cause blood clots and damage** to the walls of the smallest blood vessels (capillaries and small arteries) in the circulatory system. The blood clots can cause **injury to organs** that may lead to organ failure and death.[1]

In some cases, overactivation or dysregulation of the **complement system** can drive or worsen development of TMA. This overactivation **fuels an attack on organs and cells in the body**, including endothelial cells that line blood vessels.[1]

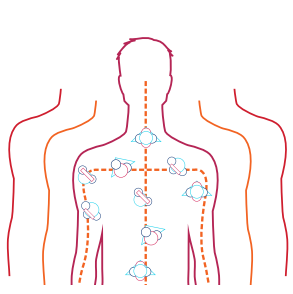




THE COMPLEMENT SYSTEM



The complement system is a part of the immune system and is **essential to the body's defence against infection**.[2]



When the system is **thrown out of balance**, or dysregulated, these proteins can **trigger a dangerous, uncontrolled cascade of reactions** that attack cells and tissues resulting in **harmful inflammation** and the **destruction of healthy cells**.[2]

Signs, symptoms and complications of TMA include:[3-5]

- Low platelet count
- Anaemia
- Thrombosis (blood clots)
- Organ damage, including kidneys, brain and heart
- Confusion
- Shortness of breath
- High blood pressure
- Fatigue

WHAT IS aHUS?

aHUS is a type of TMA that is caused by **dysregulation of the complement system,** which can occur due to genetic or environmental factors.[6]

aHUS may appear in the presence or absence of a trigger, or co-existing condition. aHUS is a rare disease that often presents suddenly with potentially severe complications and frequently develops into a **progressive, chronic condition with relapses.**[6-9]

HOW IS aHUS DIAGNOSED?

An accurate aHUS diagnosis requires first **ruling out other types of TMAs** that have similar presentations. There are no specific diagnostic tests for aHUS, which often leads to a significant delay in diagnosis.[5,10]

aHUS is considered based on a clinical evaluation of symptoms and family history. A diagnosis is ultimately confirmed by **laboratory tests**, including blood cell counts and renal function.[5]

Because the prognosis of aHUS can be poor if not recognized early, a **timely and accurate diagnosis—in addition to treatment—is critical to improving patient outcomes**.[10,11]

There remains a need for continued innovation to advance scientific understanding, increase awareness and help prevent delay in diagnosis of aHUS.

Content created by Alexion, AstraZeneca Rare Disease

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