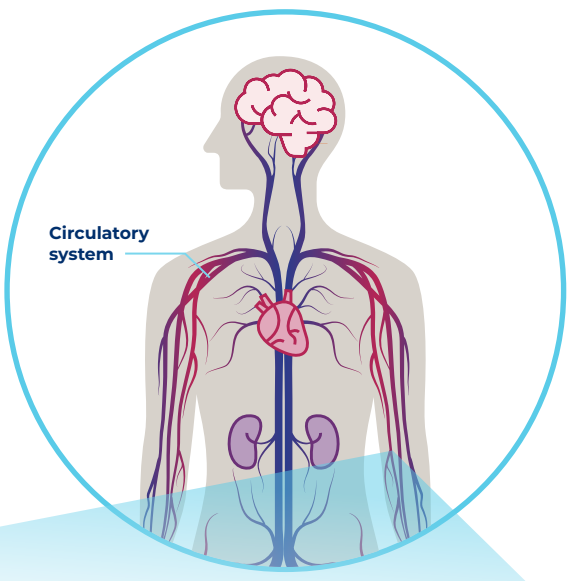
Haematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)

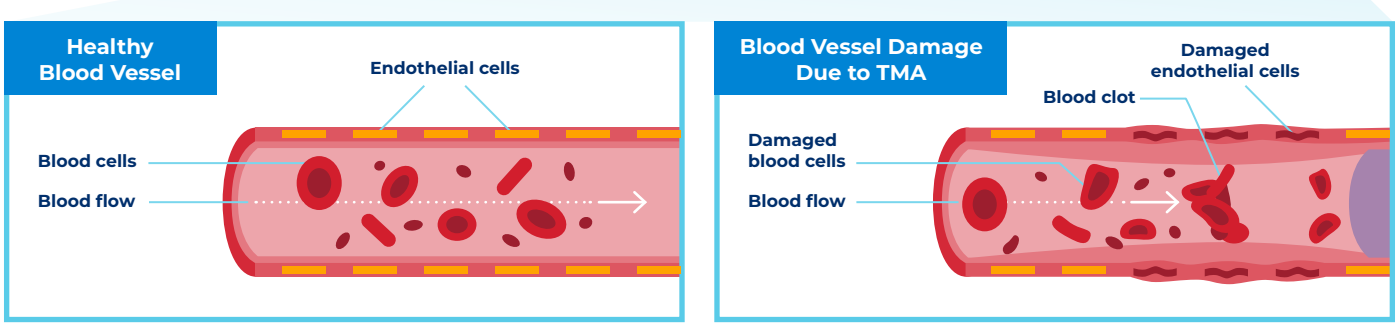
ALEXION
AstraZeneca Rare Disease

WHAT ARE TMAs?

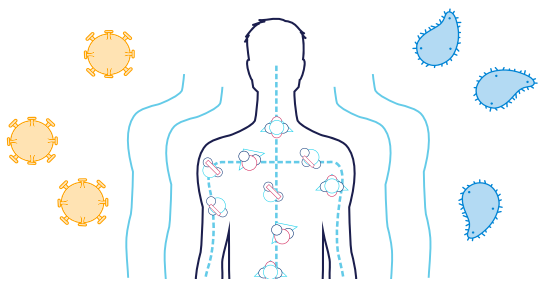
Thrombotic microangiopathies (TMAs) are a **group of severe and potentially life-threatening rare disorders that cause blood clots and damage** to the walls of the smallest blood vessels (capillaries and small arteries) in the circulatory system. The blood clots can cause **injury to organs** that may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

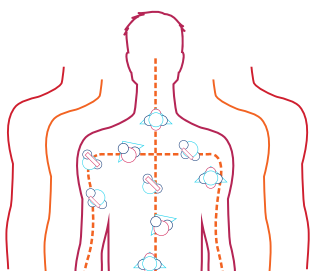




THE COMPLEMENT SYSTEM



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:[3-6]

- **Low platelet count**
- **Red blood cell abnormalities** [i.e., anaemia, fragmented red cells (schistocytes)]
- **Thrombosis (blood clots)**
- **Organ damage, including kidneys, brain and heart**
- **Confusion**
- **Shortness of breath**
- **High blood pressure**
- **Fatigue**

WHAT IS HSCT-TMA?[7]

HSCT-TMA is a **rare, severe and potentially life-threatening** type of TMA that **occurs following HSCT**, a procedure to treat some types of cancers and other diseases.

It is thought that factors associated with HSCT (including conditioning regimens, immunosuppressant therapies, infection and other complications) induce **overactivation and/or dysregulation of the complement system**, driving HSCT-TMA.

HOW IS HSCT-TMA DIAGNOSED?

HSCT-TMA symptoms can overlap with those of other conditions, which can lead to a misdiagnosis and/or a significant delay in receiving an accurate diagnosis.[7]

While there are no specific diagnostic tests for HSCT-TMA, consensus criteria recommend monitoring with **routine laboratory tests and evaluation of blood cells** under a microscope to **help make a diagnosis**.[7,8]

The prognosis can be poor if a TMA is not recognised early. There remains a critical **need for continued innovation to advance scientific understanding of the disease** and **enhance screening and detection** to improve outcomes for people living with HSCT-TMA.[7]

Content created by Alexion, AstraZeneca Rare Disease

References

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