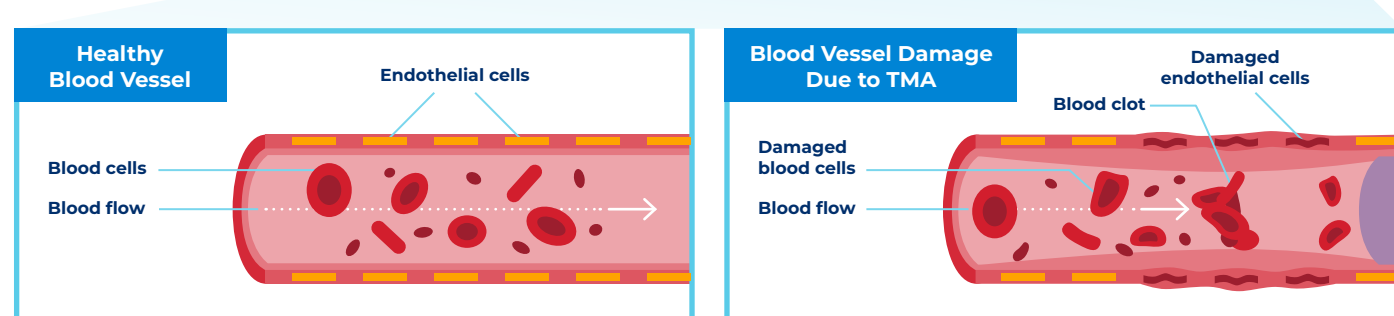
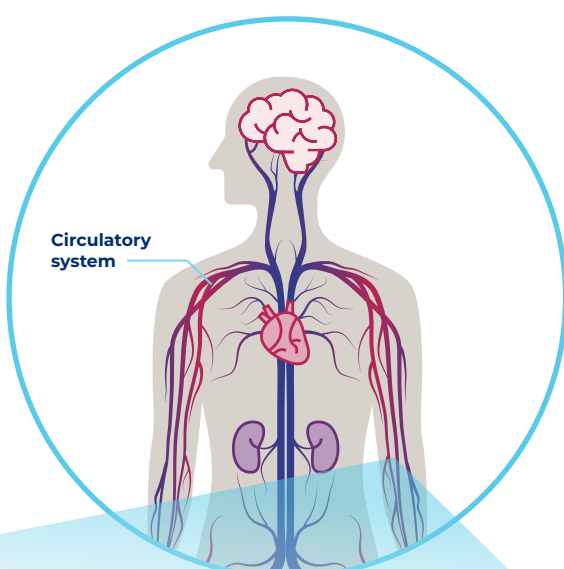


# Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)

## 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)**. The blood clots can cause **injury to organs**, that may lead to organ failure and death.<sup>1-4</sup>

In some cases, overactivation or dysregulation of the **complement system** can drive or worsen development of TMA.<sup>2,4</sup> This overactivation **fuels inflammation and an attack on organs and cells in the body**, including endothelial cells that line blood vessels.<sup>2,5</sup>



## Signs, symptoms and complications of TMA include:



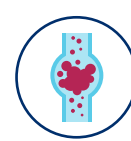
**Organ damage, including kidneys, brain and heart**<sup>3,4</sup>



**Shortness of breath**<sup>5,6</sup>



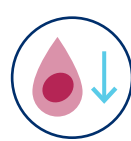
**High blood pressure**<sup>4,6,7</sup>



**Thrombosis (blood clots)**<sup>8</sup>



**Low platelet count**<sup>1</sup>



**Anemia**<sup>1</sup>



**Fatigue**<sup>5</sup>



**Confusion**<sup>9,10</sup>



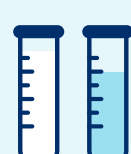
**Bruising**<sup>5</sup>

## WHAT IS HSCT-TMA?<sup>11</sup>

Hematopoietic stem cell transplant-associated-thrombotic microangiopathy (HSCT-TMA) is a **type of TMA triggered by HSCT**, a procedure to treat some types of cancers or other diseases. This rare and severe condition can present following HSCT.

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?<sup>11</sup>



HSCT-TMA symptoms can overlap with other conditions, which often leads to a significant delay in diagnosis.

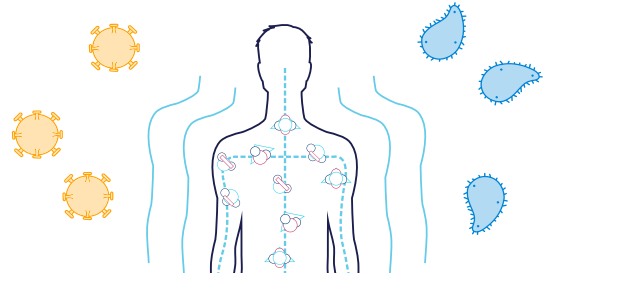
While there are no specific diagnostic tests for HSCT-TMA, routine **blood tests and evaluation of blood cells** under a microscope can **help make a diagnosis**.

Because the prognosis can be poor if not recognized early, there remains a critical **need to advance the scientific understanding of HSCT-TMA**.

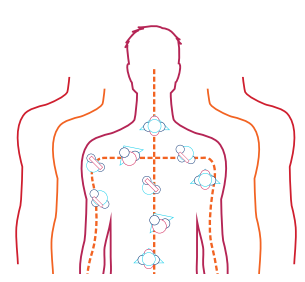
Increased awareness of the disease and patient experience has the potential to help simplify and speed diagnosis.



## THE COMPLEMENT SYSTEM



The complement system is a part of the immune system and is **essential to the body's defense against infection**.<sup>12</sup>



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**.<sup>13</sup>

## WHAT ROLE MAY COMPLEMENT INHIBITION PLAY IN TREATING HSCT-TMA?



**Alexion's leadership in complement inhibition** has set the course for the continued study and development of **innovative treatments for rare diseases**.

As complement may play a role in driving or worsening HSCT-TMA, Alexion is investigating complement inhibition as a potential treatment for this condition. Through this research, Alexion hopes to **improve the diagnostic and treatment journey for patients and their caregivers**.

## WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION?



Alexion is conducting an ongoing **Phase 3 clinical trial in HSCT-TMA** to investigate the safety and efficacy of early treatment with a **terminal complement inhibitor** (by blocking the C5 protein).



Alexion has demonstrated an **unyielding commitment to unlock the potential of the complement system** and continues to pioneer innovations for people living with rare diseases.

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