WHAT ARE TMAs?

Thrombotic microangiopathies (TMA) are a group of rare and potentially life-threatening disorders that cause blood cells and blood vessels damage leading to low levels of platelet and red blood cells. The blood cells can clump together and may block blood flow to vital organs and tissues. The blood clots can cause injury to organs, but may also interact with immune system responses and trigger a dangerous, uncontrolled cascade of reactions that attack cells and tissues resulting in organ failure, including kidneys, brain and heart. In some cases, overactivation of inflammation can drive or worsen the damage leading to organ failure and death. TMA can be divided into two types: hemolytic uremic syndrome (HUS) and thrombotic thrombocytopenic purpura (TTP).

WHAT IS HSCT-TMA?

Hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA) is a type of TMA triggered by HSCT (hematopoietic stem cell transplant). It is a rare and severe condition which can present following HSCT and include signs/symptoms suggestive of a connective tissue disorder, infection, and/or other complications related to HSCT (including conditioning regimens, immunosuppressant therapies, infection and other complications).

HOW IS HSCT-TMA DIAGNOSED?

HSCT-TMA symptoms can overlap with other conditions, which often leads to a long and difficult diagnostic journey for patients and their caregivers. While there are no specific diagnostic tests for HSCT-TMA, certain blood tests can help rule out other causes. Significant organ damage or failure, including kidneys, brain and heart, can result from HSCT-TMA. Increased awareness of all patient care teams can help make a diagnosis. Routine blood tests can also help rule out other diagnoses.

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

Complement inhibition, a potential treatment for HSCT-TMA, may play a key role in reversing TMA and helping resolve organ failure, including kidneys, brain and heart. Through research, Alexion is investigating the safety and efficacy of early treatment with a complement inhibitor (by blocking the C5 protein). This innovative treatment could potentially have a positive impact on the patient's future and improve their health outcomes.

WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION?

Alexion is conducting an ongoing Phase 3 clinical trial in HSCT-TMA, the first-of-its-kind to evaluate a complement inhibitor (by blocking the C5 protein) in HSCT-TMA. Alexion is also evaluating other potential treatment options for HSCT-TMA, including a potential role for complement inhibition. This innovative, rare disease-focused therapeutic strategy is an important step toward improving outcomes for people living with rare, chronic diseases.