aTTP FACT SHEET: What you need to know

Acquired Thrombotic Thrombocytopenic Purpura (aTTP) is a serious and rare blood disorder with life-threatening medical effects that can be fatal even with standard treatments available.

The medical community has characterized an aTTP crisis as a true medical emergency requiring immediate treatment. aTTP is an autoimmune disorder that results in the deficiency of the ADAMTS13 enzyme causing small blood clots to form throughout the body thereby preventing oxygen-rich blood flow to critical organs. Damage to critical organs happens quickly, is unpredictable, life-threatening and can be irreversible. While some aTTP patients may only face one episode in their lifetime, others can face numerous life-threatening episodes with no knowing of when it may happen again.

TTP is a rare disorder that afflicts 2-6 people per million[1], and each TTP episode is ALWAYS a matter of life and death.

An aTTP relapse can cause lengthy hospitalizations, disable, or even be fatal to an aTTP patient. Many patients stay an average of 9.7 days in the intensive care unit and an average of 14.4 days in hospital.[1]

Without effective and timely treatment, 95% of patients succumb to the disease; however, with treatment 80% - 90% of aTTP patients enter into remission[2]. 30% of these patients entering remission will relapse.[3]

aTTP can strike people of all ages, but usually young women. Many of these women have young families and promising careers. When a TTP episode occurs, immediate medical intervention is required. During this time up to 20% of patients die and others are left with life-altering complications from stroke, heart attack etc.[4]

With proper diagnosis and timely access to care, TTP risks can be reduced.

The current standard of care for aTTP is plasma exchange and immune suppression. Plasma exchange is a frightening, risky, and unpredictable treatment. Utilization of this untargeted treatment dates back to the 1980s. The treatment replaces a patient's blood plasma in an attempt to rebalance enzyme levels and remove antibodies. This untargeted treatment is repeated at least daily and then tapered-off as tolerated. Sudden life-threatening flares during tapering, or soon after, is not uncommon and requires the process to start again with daily treatments. Plasma exchange can only be

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[1] Scully M, Cataland SR, Peyvandi F, et al. Caplacizumab treatment for acquired thrombotic thrombocytopenic purpura. N Engl J Med. 2019;380:335–346 DOI: 10.1056/NEJMoa1806311

[2] Pavenski K et al. Efficacy of Caplacizumab in patients with aTTP in the HERCULES study according to initial immunosuppression regimen. Blood. 2019;134 (Supplement 1): 2365

[3] [2] Kremer Hovinga J et al. Survival and relapse in patients with thrombotic thrombocytopenic purpura. Blood 2010; 115(8):1500-1511https://doi.org/10.1182/blood-2009-09-243790

[4] Pavenski K et al. Efficacy of Caplacizumab in patients with aTTP in the HERCULES study according to initial immunosuppression regimen. Blood. 2019;134 (Supplement 1): 2365

administered to hospital in-patients and may require weeks and sometimes months of hospital stays. **For the duration of this time, the patient remains at risk of severe complications and death**.

If advancement in treatments reduce the risk of death/irreversible consequences from each TTP crisis, patient's mental health during remission will improve. The uncertainty of the severity and timing of the next relapse brings about real mental stress. A high prevalence of PTSD and depression in TTP survivors has been reported and a study found that 80.8% of individuals with TTP have mild depressive symptoms, compared 10.5% found in the general population.[5]

There is hope: Caplacizumab is a new treatment that protects patients from blood clots while standard therapies kick-in.

Caplacizumab is indicated for treatment of aTTP that, when added to the standard of care, is proven to prevent blood clots from forming in the body. This allows patients to recover from aTTP episodes quicker, and with a lower risk of damage to organs and other long-term disabilities associated with aTTP.

Caplacizumab is the **first targeted treatment** that prevents the formation of blood clots, buying time for patient's treatments to work while being protected from additional long-term health consequences.

We are seeking access to treatment for those who need it most.

Caplacizumab was granted a priority review and **approved by Health Canada** in March 2020.

- In September 2020, CADTH's Common Drug Review issued a negative recommendation for caplacizumab citing issues with the clinical trial design. Canada is an outlier when it comes to coverage for caplacizumab: the treatment is currently covered for patients with aTTP in peer countries such as United States, Austria, Belgium, Denmark, Netherlands, Finland, Italy, and the UK.
- Caplacizumab was recommended for reimbursement by the National Institute for Health and Care Excellence (NICE) in the UK with the same clinical study submission sent to CADTH. CADTH and NICE have a long track record of working together, including supporting efforts for Early Scientific Advice for new and breakthrough therapies
    - NICE recognized that the benefits of the drug outweighed some of the questions it had in its cost-effectiveness estimates including the reduction of utilizing scarce resources such as plasma or ICU beds.

We are looking to develop a path forward and seeking the provincial government's support for aTTP patients by providing access to caplacizumab for patients that would benefit most from treatment – namely those who do not respond to current treatments and/or those deemed appropriate for treatment by their physician.

[5] Chaturvedi S, Oluwole O, Cataland S, McCrae KR. Post-traumatic stress disorder and depression in survivors of thrombotic thrombocytopenicpurpura. Thromb Res. 2017 Mar;151:51-56. doi: 10.1016/j.thromres.2017.01.003. Epub 2017 Jan 6. PMID: 28113083