

Date

Hon. (Minister)
(Ministerial Title)
(Address 1)
(Address 2)
City, Province
Postal Code

Dear Minister (name),

I am writing to you today on behalf of (NAME), a local constituent of mine who met with me to discuss acquired Thrombotic Thrombocytopenic Purpura (aTTP), a rare, unpredictable, autoimmune blood disorder that presents as a **true medical emergency**. aTTP is characterized by small blood clots throughout the body that prevent oxygen rich blood flow to critical organs leading to irreversible disability and/or death. More specifically, my constituent shared their concerns around their inability to access the only therapy in Canada for aTTP, caplacizumab, under our province's public drug plan that can act immediately to shield them from irreversible life-altering complications and/or death.

The medical community has characterized an aTTP crisis as a true medical emergency because damage to critical organs from the small blood clots happens quickly, is unpredictable, life-threatening, and can be irreversible. The first symptom of aTTP that brings a patient to an ER could be as serious as a stroke. aTTP can strike people of all ages, and can show a variety of symptoms from fatigue, fever and bleeding to neurologic symptoms and bruising. It is estimated that only 2-6 per million people per year are diagnosed with aTTP, making it extremely rare and difficult to diagnose. Even with timely treatment and access to care, up to 20% of patients in each aTTP crisis still die, another 10% suffer irreversible and life-altering complications. Up to 30% of those diagnosed with TTP will unpredictably relapse.

The standard of care in Canada is immunosuppressant drugs combined with plasma exchange, where patients' blood plasma is replaced. 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 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. Even then, some patients do not respond to treatment. Statistics show that most patients stay an average of 9.7 days in the ICU and average of two weeks in hospital.

Without effective treatment, aTTP patients and physicians are forced to wait with bated breath minute by minute (often intubated in the ICU), not knowing if the therapy will work in time, and whether the next clotting complication could be fatal. An aTTP relapse can cause another lengthy hospitalization, further complications, or even be deadly.

While in remission, the uncertainty of the severity and timing of the next relapse brings about real mental stress. If advancement in treatments reduce the risk of death/irreversible consequences from each TTP crisis, patient's mental health during remission will improve. 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

Last year, Health Canada approved caplacizumab, the first therapy available in Canada to specifically treat aTTP. Caplacizumab is proven to prevent blood clots from forming in the body, and in effect, shielding patients from clotting complications while standard therapies have the chance to start working. Caplacizumab allows more patients to recover from aTTP, to do so faster, and with a lower risk of damage to organs and other long-term disabilities.

Unfortunately, the Canadian Agency for Drugs and Technology's (CADTH) Common Drug Review and INESSS issued a negative recommendation for caplacizumab in September 2020, citing concerns with the design of the clinical trials conducted for the drug. This decision comes despite a Health Canada approval, there being no new treatment for aTTP in 25 years, and the fact that the drug is recommended and funded for patients in numerous peer countries, including the United States, Austria, Belgium, Denmark, Netherlands, Finland, Italy, and the UK. The National Institute for Health and Care Excellence (NICE) in the UK have a long track record of working with CADTH and despite sharing the same concerns as CADTH, NICE recognized that the benefits of the drug outweighed the questions it had. NICE knew that caplacizumab could not only help aTTP patients but save scarce resources such as plasma or ICU beds, both of which (province) should consider as COVID-19 has limited hospital budgets and global plasma levels remain low and in demand.

(NAME) is 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.

Thank you for your consideration of this constituent request – I look forward to your response.

Sincerely,

MPP (name)

cc. **CONSTITUENT NAME**
 CONSTITUENT CITY
 CONSTITUENT EMAIL