



Answering TTP Foundation  
www.AnsweringTTP.org  
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February 27, 2023

Deputy Minister Dicerni,  
ADM, Health Programs and Delivery, General Manager of OHIP, and Executive Officer of Drugs  
Ministry of Health  
100 University Ave Fl 9  
Toronto, Ontario  
M5J 2Y1, Canada

Dear Deputy Minister Dicerni,

The Answering TTP Foundation represents patients with acquired Thrombotic Thrombocytopenic Purpura (aTTP), a rare and deadly blood disease **characterised by dangerous small blood clots that form throughout the body during a crisis episode**. It is these blood clots that make this disease fatal without immediate treatment, and even with treatment up to 20% of patients in an aTTP crisis will die of the disease, and the majority of those who survive are left with complications and disabilities from the small blood clots for life. Up to 30% of patients will face these devastating statistics again in subsequent unpredictable relapses.

**On behalf of Canadians who have survived an episode of aTTP and the families of those patients who have died already from aTTP, the Answering TTP Foundation is urging you to immediately add caplacizumab to the provincial formulary.**

Caplacizumab is the first targeted treatment for aTTP. It is designed to stop the small blood clots thereby keeping our community safe during relapse. This treatment gives our community life-saving hope, and piece of mind. By adding caplacizumab to Ontario's Public Drug Formulary, we can save the lives of about 10 Ontarians per year, and protect another 50 or so per year from devastating long-term consequences from blood clots.



In January 2021, Lorraine Wigston suffered a second relapse of TTP after contracting COVID-19 from her dying father who had tested negative days before. Despite receiving standard TTP treatment, Lorraine's case seriously worsened and she became very scared that maybe she would not dodge the bullet this time. Caplacizumab was added to her treatment regime and she was released from the hospital two weeks later, requiring half the time in hospital as her first two TTP episodes. Lorraine is grateful to have been provided the opportunity to receive caplacizumab.

Caplacizumab been shown to save refractory patients, and help patients recover faster, and with less damage to their vital organs – damage that can accumulate with each relapse of aTTP. Caplacizumab is not an ongoing treatment, it is only required during a crisis as an immediate defence to stop the dangerous clotting. Once standard treatments correct the underlying disease mechanism, this barrier of protection is not longer needed, and is stopped (we are only asking for only weeks of treatment during a crisis).



Selena, a 19 year-old, had been in hospital for almost two weeks, and had suffered multiple strokes. Her case was grave. Then, she was given caplacizumab. Overnight she stabilized.

Caplacizumab has been approved by Health Canada, the FDA, and similar regulatory bodies around the world. Most other jurisdictions in the world – including the United States, Austria, Belgium, Denmark, Netherlands, Finland, Italy, and the UK – have provided access to caplacizumab. But in Canada, the failure of the Canadian Agency for Drugs and Technologies in Health’s (CADTH’s) methodology to adequately evaluate the performance of caplacizumab in the rare disease landscape has put the lives of Ontarians at risk. Please see the Foundation’s feedback to CADTH’s latest draft negative recommendation attached to this correspondence.

Despite the negative CADTH review, our community remains determined to ensure access to all TTP patients in need. Real World Evidence (RWE) generated in both Canada and in peer countries has clearly demonstrated the efficacy of caplacizumab.



Yhulan Walters, a child and youth worker in Toronto, was rushed by ambulance to hospital with her 3rd TTP relapse in critical condition. She was intubated for a week in the ICU. Only after caplacizumab was added to her treatment regiment did her case turn around.

Patients need access to caplacizumab because it is the first targeted treatment that addresses the formation of blood clots that are the cause of organ damage, and thus prevents the long-term disabilities and death associated with aTTP. It helps get patients out of the ICU and the hospital faster.

Caplacizumab is approved by Health Canada, is strongly supported by the patient community, and its use is encouraged by the medical experts in the field. Specifically, current clinical practice guidelines state:

**“Caplacizumab must be used as a first-line therapy in severe TTP”** (Strength of recommendation: Strong) -*Expert Statement on the ICU Management of Patients with Thrombotic Thrombocytopenic Purpura.* <sup>9</sup>

**“For patients with iTTP experiencing an acute event (first event or relapse), the panel suggests using caplacizumab over not using caplacizumab” -International Society on Thrombosis and Haemostasis Guidelines for Treatment of Thrombotic Thrombocytopenic Purpura.<sup>10</sup>**

Adding caplacizumab to the provincial formulary will mean that Canadian physicians would be able to provide the recommended evidence-based care for their patients consistent with international treatment guidelines.

In January 2021, 35-year-old Stacy Kertzer’s worst nightmare during the COVID-19 pandemic came true. Stacy was admitted to hospital with her second TTP relapse. For 11 days she did not improve despite standard treatment and remained at serious risk of major complications. Then, she says she was given a miracle in the form of caplacizumab. Incredibly, 4 days later she was well enough to be released from hospital.

Failing the immediate ability to provide access, we would appreciate a meeting to discuss access to this life-saving treatment in Ontario at your first opportunity.

Sincerely,



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