



atypical
Hemolytic
Uremic
Syndrome

Supporting patients and families
living with aHUS

February 3, 2015

Hello Minister Sousa, I'm Michael Eygenraam and I am here today on behalf of aHUS Canada.

As I read this statement, about 30 people in Ontario have a very rare, life-threatening, genetic disease, known as atypical Hemolytic Uremic Syndrome also known as aHUS. You might recall hearing about aHUS since you, Minister Sousa, had a recent conversation with your friend Yvonne Williams regarding a patient, Toni Vernon.

aHUS is caused by chronic, uncontrolled activation of the complement system, a part of the body's natural immune system. As a result, the immune system attacks the body's healthy cells, which can cause abnormal blood clotting and blood vessel damage.^{i,ii} The presence of blood clots causes damage to organs, leading to heart attack, stroke, kidney failure and death.ⁱⁱⁱ

Acquiring aHUS happens without warning. In 2002, I thought I had the flu but became really sick and I was rushed to emergency where I was misdiagnosed with a similar disease to aHUS. Despite receiving many plasma treatments, I lost both kidneys and needed dialysis. In 2006, my wife donated a kidney but the transplant failed as the disease returned despite more plasma. In 2011, I was properly diagnosed with aHUS and was told I could not receive another transplant unless aHUS was controlled.

Plasma Therapy currently treats symptoms of aHUS. It is not studied or proven to be safe or effective.^{iv} Despite plasma therapy, 65 percent of patients die, need dialysis or have permanent kidney damage within the first year of diagnosis.^v Soliris is currently the only Health Canada approved life-saving drug that exists for aHUS.^{vi} Soliris helps to prevent and restore the loss of kidney function.^{vii} It has also revolutionized the management of this disease by opening up the possibility of kidney transplants for patients^{vii} like Toni Vernon and I. Toni is also on dialysis and found a donor match, but her doctor won't perform the transplant because without Soliris, chances are very high that her kidney will fail again. aHUS and dialysis cause severe fatigue and many other side effects.^{viii} This has prevented us from working and significantly affects our quality of life.

We've met with many government officials and are very concerned by what we have heard from some. They have made incorrect statements. One actually told us that Plasma is a proper treatment for aHUS. If Plasma were a proper treatment I wouldn't have lost my kidneys, I wouldn't have lost my wife's kidney, and I would be able to work. We have heard the safety of Soliris called into question, even while Health Canada approved it for safety and Ontario is supplying it to PNH patients.

Yes, the drug is expensive. It averages \$500,000 a year per patient. But if you look at the small, finite number of patients and what is currently being spent on unproven treatments, you will see that it is affordable. How much does plasma therapy cost? How much does dialysis cost? How much does routine hospital visits cost because I'm not on a proper treatment? How much is it costing every year I can't work, every year I can't pay income tax?

According to the Canadian Organization for Rare Disorders, "In Canada, less than 1% of our public drug budget is for rare diseases. In Europe, the impact in countries with the very best access is still only about 2.5% – 3.5% of drug costs."^{ix} While Soliris is expensive, by denying aHUS patients this drug we are jeopardizing their health and putting a price on people's lives. While we agree that the government should have some say when it comes to high cost treatments, we believe that physicians know best which treatments patients need.

Johann Kerlow's physician was denied funding of Soliris twice. After being hospitalized with kidney failure and multiple pancreatic attacks, the government made Soliris accessible to her for six months. Because of the delay, she needs dialysis. Why do we need to be at death's door in order to receive proven treatment? Of the approximately 30 patients in Ontario, half of them are children.^x One child, Joshua de Bortoli, was fortunate to be part of a clinical trial for Soliris which saved his life. Another child lost parts of limbs, intestines and complete kidney function.

We are told that public funding of Soliris in Ontario has not been granted because there is a lack of clinical data. In fact, clinical information has been provided to the Ontario government - by aHUS Canada, medical experts, and the manufacturer - which demonstrates that Soliris has significant improvement in patient outcomes over Plasma therapy. With Soliris, 90 per cent of patients have normal blood levels, showing the disease is under control.^{xi,xii} Studies show that patients on Soliris can discontinue plasma and dialysis therapies, have improved kidney function, normalized blood levels, reduced blood vessel damage and decreased risk of blood clots.^{xi} Other jurisdictions throughout the world, including the Province of Quebec, already make Soliris accessible through public funding based on the same clinical data. Just last week in England, the National Institute for Health and Care Excellence issued its final guidance on Soliris, recommending funding for all aHUS patients there.^{xiii} It called Soliris a "significant innovation for a disease with a high unmet clinical need."^{xiii} A clinical need clearly not met by plasma therapy.

We are told that there is a process that needs to be followed. But, with all due respect, this has been a very long process and lives are at risk. It's been 2 years since the Notice of Compliance was issued by Health Canada for Soliris, 18 months since the Common Drug Review's recommendation and 13 months since two CED recommendations were made.

This issue is not political - it has support from all parties. This is about doing the right thing for Ontarians. We are asking you, as an elected official and as the Minister of Finance for this province, to help us. I'm here today to say that this is a matter of life and death and that you can help Ontario by making the funds available for this important medication that will improve and save patient's lives.

Drugs for rare diseases are usually expensive and this should not be used as a reason for not funding them. Not funding is giving priority to patients with common diseases over those with rare diseases. No one chooses to have a rare disease.

Help us live healthy lives.

Help us save organs and limbs.

Help us save lives.

References

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