atypical Hemolytic Uremic Syndrome

aHUS CANADA

Supporting patients and families living with aHUS

TWO ONTARIANS LIVING WITH ULTRA-RARE DISEASE URGE GOVERNMENT OFFICIALS TO PROVIDE EQUITABLE ACCESS TO LIFE-SAVING TREATMENT

- Ontario expert is leading international aHUS patient registry with goal of improving care, but province not funding only approved treatment -

TORONTO, ON – February 27, 2014 – To mark Rare Disease Day 2014, two patients affected by the same ultra-rare disease, atypical Hemolytic Uremic Syndrome (aHUS), shared very different stories with government officials at Queen's Park today. The reason for their visit was to raise awareness of the urgent need for publicly-funded access to Soliris® (eculizumab), the first and only pharmaceutical treatment option for this chronic and life-threatening disease.

On March 2013, Soliris was approved by Health Canada as safe and effective for use in children and adults with aHUS, based on a strong body of clinical evidence. In fact, since then, additional studies have been published in leading peer-reviewed journals, such as the New England Journal of Medicine, reinforcing international expert consensus supporting the use of Soliris as the new standard of care for children and adults with aHUS. Unfortunately, the Ontario government has restricted access to the life-saving treatment by refusing to fund it. Unless they were part of the clinical trial that led to its approval in Canada, only those patients with private drug coverage have the opportunity to access Soliris.

Inequitable access to treatment leads to vastly different outcomes

Almost two years ago, Joshua DeBortoli, 12, of Vaughan, Ontario, was admitted to The Hospital for Sick Children where he was admitted and stayed for five weeks, including one week in the coronary care unit (CCU) unit, until he was diagnosed with aHUS. He endured five days of continuous vomiting and blood testing several times per day, as well as numerous blood transfusions for weeks and internal bleeding, and he was intubated and put on oxygen. Josh also developed a blood clot and kidney failure, which led to hours of daily dialysis and plasmapheresis to combat the debilitating and life-threatening symptoms of the disease. When Joshua's doctor enrolled him in a clinical trial for Soliris, his health dramatically improved and his kidney function was restored. The DeBortoli family was incredibly relieved to see Joshua's disease under control. They knew he was lucky to have been diagnosed in the right place, at the right time.

"When I was really sick, my biggest wish was to be back at school with my friends. Since I started getting Soliris for my aHUS, my wish came true. I can do the things I love, like karate, playing soccer, baseball and the drums, and I haven't gotten sick in the last two years," said Joshua. "I feel healthy and I hope that other people with aHUS can get Soliris, so they can be healthy, too."

Michael Eygenraam of Brampton, Ontario, a 49 year-old father of two teenagers and board member of aHUS Canada, was one of the unlucky ones. When he first became sick in 2002, he was misdiagnosed with a disease which has similar symptoms to aHUS and, within six months, had lost both his kidneys. In order to survive, Michael began hemodialysis five times per week. Three years later Michael's wife, Margriet, donated one of her kidneys but, within mere weeks, the transplant began failing and he had to go back on dialysis. Dialysis is a life-long therapy which has a significant impact on Michael's quality of life and causes pain, yet it does not address his underlying disease. In 2010, a diagnosis of aHUS was finally confirmed, explaining Michael's transplant failure. Currently, aHUS makes him feel unwell, causes severe fatigue and leaves him unable to work.

"I am asking the Ontario government for a real chance at a normal life. I won't be a candidate for another kidney transplant until I'm on a treatment that will control my disease, and the only treatment proven to do that is Soliris. Without it, any donated kidney would fail again," explained Eygenraam. "If the Ontario government continues to deny public funding for Soliris, my life will be cut short and that is not something my family or I can or will accept."

Quebec makes evidence-based funding decisions; others follow flawed review process
When Health Canada approved Soliris, patients and their loved ones across the country saw their first glimmer of hope in the face of this devastating disease.

In July 2013, hope for patients like Michael was lost when the Common Drug Review (CDR) recommended that this life-saving treatment not be funded by provincial and territorial public drug plans – a decision resulting from a review process that is limited in scope for evaluating drugs for rare diseases. The CDR recommendation has been heeded by all but one province to date. Quebec, which does not participate in the CDR, is currently the only province to provide aHUS patients with public funding for Soliris through its exceptional drug access program, *Patient d'exception*. Additionally, publicly funded access to Soliris is provided to aHUS patients in 30 countries around the world, including the U.S. and Japan, as well as nations in the E.U. and Latin America.

"I am pleased that the Quebec government has recognized international expert consensus supporting the use of Soliris as the new first-line standard of care for children and adults diagnosed with aHUS," said Dr. Anne Laure Lapeyraque, a Montreal-based pediatric nephrologist and medical advisor to aHUS Canada. "It is my hope that the other provinces and territories follow Quebec's lead and provide public funding of Soliris for aHUS patients to ensure equal access to this important treatment across the country."

First aHUS patient registry led by Ontario expert strives to improve care, outcomes
As patients anxiously await public funding for Soliris, Dr. Christoph Licht, a world-leading aHUS researcher and pediatric nephrologist at The Hospital for Sick Children, is doing all that he can to ensure aHUS patients are followed by experts and benefit from best practices. aHUS Canada congratulates Dr. Licht and The Hospital for Sick Children for their leadership in chairing the first global aHUS patient registry. In addition to tracking and assessing the long-term impact of aHUS on children and adults living with this ultra-rare disease, the registry will also monitor the safety and efficacy of Soliris and other emerging therapies, with the goal of improving care, outcomes and quality of life for aHUS patients.

"Through this global registry and our practice at SickKids, our team is committed to ensuring that aHUS patients in Ontario and worldwide receive the best possible care for this potentially life-threatening disease," said Dr. Licht, who is also a medical advisor to aHUS Canada. "As safe and effective aHUS treatments are approved, it is critical that patients have access to them to ensure the best possible outcomes. We support aHUS Canada's mission to advocate for access to the best care and treatment for this community."

Lack of access to treatment forces patients to rely on substandard therapies
Prior to the approval of Soliris, the management of aHUS relied on plasma infusion and plasma exchange therapies, which have not been clinically proven to be safe or effective in aHUS.[i] Studies show that within the first year after diagnosis, 65 per cent of all aHUS patients die, require dialysis, or have permanent kidney damage, despite plasma exchange or plasma infusion.[ii] The majority of aHUS patients progress to end-stage kidney failure within three years of diagnosis.[iii,iv] Without Soliris, kidney transplant is not a viable option for the vast majority of aHUS patients, since the disease recurs in 60 per cent of cases, and transplant failure occurs in 90 per cent of cases.[v]

"By ignoring the solid evidence and expert opinion on Soliris, the Ontario government is forcing us to rely on substandard, unproven therapies and is exposing us to life-threatening infections and allergic reactions, permanent organ damage and death," concluded Eygenraam. "We know that Soliris can significantly improve

our health and quality of life, and we implore the government to give back our hope for a normal life by providing immediate funding for Soliris."

About aHUS

Affecting between 60 and 90 children and adults in Canada, aHUS is a very rare, chronic and life-threatening genetic condition, which leaves a part of the immune system (known as the complement system) uncontrolled and always active. As a result, the immune system attacks the body's unhealthy and healthy cells which can cause blood vessel damage, abnormal blood clotting[vi,vii] and progressive damage to the major organs, leading to heart attack, stroke, kidney failure and death.[iii]

About aHUS Canada

aHUS Canada was formed in November 2012 to support Canadian patients and families living with aHUS. In addition to establishing a Canadian aHUS community, the group is committed to building public awareness and understanding of aHUS and advocating for the best possible care and treatment for patients. For more information, please visit www.ahuscanada.org.

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