atypical Hemolytic Uremic Syndrome

aHUS CANADA

Supporting patients and families living with aHUS

CHILDREN AND ADULTS AFFECTED BY ULTRA-RARE DISORDER URGE GOVERNMENTS TO FUND LIFE-SAVING MEDICATION

-International study published in leading medical journal underscores efficacy of Soliris® -

TORONTO, ON – July 25, 2013 – Only a few months ago, a very small group of Canadians affected by atypical Hemolytic Uremic Syndrome (aHUS) rejoiced upon hearing the news that the drug Soliris® (eculizumab) had been approved for a second ultra-rare disorder – the one that threatens their lives. Today, these same patients – approximately 60 children and adults in Canada – fear access to this life-saving treatment may be delayed or restricted due to a recommendation made by the Common Drug Review (CDR) to provincial and territorial drug programs not to fund the medication.

Soliris is the first and only pharmaceutical treatment approved for aHUS in children and adults, and is hailed by experts worldwide as a critical breakthrough in altering the course of the disease. It has been shown to significantly improve patients' health and quality of life.[i] In clinical trials, Soliris was proven effective in preventing blood vessel damage and abnormal blood clotting,[ii,iii] leading to remission and significant improvement in kidney function.[iv,v] Soliris has also allowed patients to discontinue dialysis and plasma exchange therapies.[i]

"Before starting Soliris, I experienced extreme weakness, constant fatigue, heart problems, and life-threatening reactions to frequent plasma exchange treatments that prevented me from working and providing for my young family – a very difficult reality for anyone to face," says Brian Tjepkema of Abbotsford, B.C., who was diagnosed with aHUS in 2010. "I have been taking Soliris for a year now, I no longer live in fear of suffering a deadly stroke, and my health has almost returned to normal. I am grateful that I can once again be the husband and father I've always known I can be."

Recently-published international evidence supports efficacy

The results of a recently-published clinical trial co-led by one of the leading international aHUS experts, Dr. Christoph Licht, coupled with the existing body of evidence for Soliris show that the treatment not only has the potential to save the lives of children and adults affected by the disease, but also to improve health-related quality of life. According to the international study,[vi] which appeared in the New England Journal of Medicine in June 2013, Soliris was able to restore control of part of the patient's immune system known as the complement system. Whereas, the traditional treatment of plasma infusion/exchange only achieved partial control. Patients in the study treated with eculizumab were able to discontinue plasma infusion/exchange and dialysis therapies. Results also showed that Soliris improved kidney function, reduced blood vessel damage and decreased the risk of blood clots.

"For the first time, we have a demonstrated treatment for aHUS that directly and effectively targets the underlying cause of this devastating disease, bringing a life-altering benefit to patients," says Dr. Christoph Licht, Associate Professor of Pediatrics, Division of Nephrology at The Hospital for Sick Children, Toronto, and Medical Advisor to aHUS Canada. "My international colleagues and I concur that Soliris is a critical tool that can have a positive impact on patients' lives."

Families hope funders look beyond CDR

In 2009, Soliris was first approved in Canada for another ultra-rare blood disease called paroxysmal nocturnal hemoglobinuria (PNH), and despite receiving a negative CDR recommendation, it is now accessible to patients across the country through private and public drug plans. Families like the DeBortolis from Vaughan, Ont. who see the benefits of Soliris in their 11-year-old son Joshua, can only hope that provincial and territorial drug plans will once again base their funding decisions on the advice of aHUS experts and the strength of clinical evidence, rather than a review process ill-designed to get medications for rare disorders to patients quickly.

"We are fearful that any delay in funding could mean that patients' lives will remain needlessly at risk. We know how fortunate our son was to receive Soliris when he did, it's heartbreaking to think that other children, or adults, may not be so lucky," says Sonia DeBortoli, a member of the aHUS Canada Board of Directors. "Still, we have to be optimistic that governments will look beyond CDR and act quickly to provide publicly-funded access to this life-saving treatment for aHUS patients, before any more damage is done to life, or limb."

About aHUS

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 [vii,viii] and progressive damage to the major organs, leading to heart attack, stroke, kidney failure and death.[iv]

The management of aHUS has relied on plasma infusion and plasma exchange therapies with variable results.[v] These lifelong therapies are costly, painful and time-consuming, and have not been studied or approved for the treatment of aHUS.[ix] If kidney failure has already occurred as a result of aHUS, dialysis is required, though it is not a curative treatment.[x] Within a year of diagnosis, over half of patients will need dialysis, will have irreversible kidney damage, or will not survive.[ix] The majority of aHUS patients progress to end-stage kidney failure within three years of diagnosis.[xi,xii]

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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