

## **THOUSANDS OF ONTARIANS SUPPORT ULTRA-RARE DISEASE GROUP'S PLEA FOR PUBLIC FUNDING OF LIFE-SAVING DRUG**

*- aHUS patients appeal to government to end two-year wait for access to Soliris -*

**TORONTO, ON – February 25, 2015** – Ontario families affected by aHUS (atypical Hemolytic Uremic Syndrome), an ultra-rare, genetic, life-threatening disease, are at Queen's Park today, to make an urgent appeal to the Government for an immediate long-term funding solution for Soliris (eculizumab) – the life-saving treatment that could benefit an estimated 30 adults and children in the province. Buoyed by thousands of petition signatures showing support for their plea, the group is bringing awareness to the two-year-long wait for access to Soliris just days before International Rare Disease Day, recognized annually on the last day of February.

"Nearly two years after Soliris was approved by Health Canada as being the first safe and effective treatment for aHUS, we continue to appeal to the Premier and Health Minister to provide our community with urgently needed access to this treatment," said Michael Eygenraam, Vice-Chair of aHUS Canada. "aHUS Canada is very willing to work with government to find an immediate but long-term solution to this issue, as the organs, limbs and lives of our very small community depend on the province's decision to fund this life-saving medication through the Ontario Public Drug Programs."

### **Unproven, sub-standard therapies in use leave lives at risk**

Prior to the approval of Soliris, the management of aHUS relied on plasma therapies which have not been clinically proven to be safe or effective in aHUS.<sup>1</sup> Studies show that despite plasma exchange or plasma infusion, 65 per cent of all aHUS patients die, require dialysis, or have permanent kidney damage within the first year after diagnosis.<sup>2</sup> Before Soliris, kidney transplant was not a viable option for the vast majority of aHUS patients, since transplant failure occurs in up to 90 per cent of cases.<sup>3</sup>

Unlike plasma therapies, Soliris has the potential to change the course of the disease, to repair and restore kidney function in some patients<sup>4,5,1</sup> and, for the first time, offers a real opportunity for transplant in those aHUS patients on chronic dialysis.<sup>5</sup> International studies have shown that Soliris is highly effective in controlling aHUS and improving patients' quality of life.<sup>5</sup>

Three years ago, when he was 10 years old, Joshua DeBortoli of Vaughan, ON experienced the debilitating impacts and life-threatening symptoms of aHUS. When the disease attacked, Josh developed a blood clot and went into kidney failure, which led to daily plasma therapy and dialysis. Initially, his parents didn't know if Josh would survive, but once diagnosed with aHUS, he was enrolled into a clinical trial for Soliris. Soon after, Josh's full kidney function was restored, his health returned, and he was back to living like a normal little boy.

"Back when I was diagnosed with aHUS, I was very sick and my family was very worried about what would happen to me," said Josh. "Once I started Soliris, my life went back to normal. I realize how lucky I was to have access to Soliris when I did, and I want other kids and adults with aHUS to be able to get it, too."

### **Ontario still not funding Soliris despite expert consensus and strong clinical data**

International experts now recognize Soliris as the new standard of care for aHUS, and governments around the world have committed to funding it for their patients. In January 2015, aHUS patients in England joined their ranks when the UK's National Institute for Health and Care Excellence (NICE) issued its final guidance in support of publicly funding Soliris, acknowledging the treatment as a "significant innovation" in managing aHUS.<sup>6</sup> In Canada, only the province of Quebec funds Soliris for aHUS patients.

Early last fall, Markham, ON resident and mother of three, Johann Kerlow, was repeatedly hospitalized due to complications of aHUS. By mid-October, as Johann's life hung in the balance, the Ontario government agreed to provide her with temporary funding for Soliris for up to six months through Ontario's *Compassionate Review Policy*. Now, Johann and her family need the assurance that she will be able to continue to access Soliris long-term to protect her from this deadly condition.

"I was fortunate to begin receiving Soliris when nothing else was controlling my disease, and I am grateful to be alive, but my kidneys sustained so much damage while I was waiting for access to treatment that I am now fully dependent on dialysis," said Johann. "Soliris has certainly changed the quality of my life, and without it, I am fearful that my condition will begin to deteriorate again."

### **About aHUS**

aHUS is a life-threatening genetic condition which can occur at any age, and affects children and adults almost equally. In aHUS, a part of the immune system (known as the complement system) is left 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<sup>7,8</sup> and progressive damage to the major organs, leading to heart attack, stroke, kidney failure and death.<sup>4</sup>

### **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](http://www.ahuscanada.org), or visit us on [Facebook](#) and [Twitter](#).

We also invite you to check out our [YouTube](#) page to watch our new videos:

- [Patients appeal for funding of only effective treatment for life-threatening disease](#)
- [A family's urgent plea for access to a life-saving drug for an ultra-rare disease](#)

-30-

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## References

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