3rd April, 2017

Dear Legislative Council Members and Government Officer,

I am a mother of 3 with 2 children diagnosed with aHUS. Both were diagnosed when they were still babies, 10 months old and 8.

aHUS is an extremely rare chronic and life threatening genetic disease. This disease uncontrollably activates the complement system which is part of the body's natural immune system.

Most patients with aHUS will progress to end-stage kidney failure within a few years of diagnoses. These patients have relapses and death rates are high. My second child has had 3 relapses and third had 2 already. Second child's kidney has reached the end stage already. Kidney transplant is advised by the doctors but transplant will not stop the disease from recurring. Unless there is medication to stop the complement body from abnormal blood clotting and blood vessels damage then the kidney will keep on getting damage.

My second child is now 8 years old. Each relapse was not only life threatening but also caused health deterioration in the end. He had a seizure during his third relapse. He must rely on daily medication and plasma exchange.

My child has had over 5 surgeries already. Going in and out of the surgery theater, always made my son very scared, anxious and even became paranoid with what he does in his everyday life. I saw his hands trembling through smiles before going to a room to have anesthesia. He smiles because he doesn't want me to worry about him. He was acting brave. He is paranoid about leaving the house when the weather was not good like raining, cold or even cloudy. He missed a lot of activities that an eight-year-old should be doing. Basically his childhood is destroyed by aHUS. He stopped playing soccer which he enjoyed the most. He has no play dates since most of his time is spent in hospital. He once even said he hates festivals and holidays the most because he gets to spend them in the hospital.

After the first seizure, my son had short term memory loss and a dramatic change in his behavior. I remember that it took a while for him to remember who my husband and I were after he woke up. As a parent, it was heartbreaking to see that moment. He even didn't know how to speak properly after he woke up.

Hospitalization often took over half of his school year. He would often come home crying with complaints about not being able to catchup with the schoolwork. He often felt lonely because he doesn't have friends anymore. He was isolated by them.

In overseas, there is a drug called Eculizumab used for treating aHUS patients. Studies have shown that:

Eculizumab has shown greater efficacy than plasma exchange in the prevention and treatment of aHUS. Clinicians advise that patients with native or transplanted kidneys whose aHUS recurs be treated with this drug , and that treatment be initiated as early as possible for optimal recovery of renal function. Switching from plasma therapy to eculizumab has been shown to improve renal function even in patients with long-lasting and stable chronic kidney disease. In clinical trials, it has been proven effective in preventing blood vessel damage and abnormal blood clotting. In June 2013, an international study in the New England Journal of Medicine showed aHUS patients treated with eculizumab were able to discontinue plasma exchange and dialysis therapies, and saw improved kidney function, reduced blood vessel damage and decreased risk of blood clots.

Eculizumab is exactly the same drug as the drug for PNH patients that Community Care Fund is planning to subsidize. Please also consider this drug to help the need of aHUS patients.There is no reason for HA to abandon aHUS patients who suffer serious burden and awaited for suitable drug treatment.

My children with aHUS are ages of only 8 and 6 now. Hopefully they will have a long life ahead. Their life expectancy can be extended by using the drug and it's proven by other patients already. My 8 year old son has set up his goal to be a chef when he grows up. He says he wants to cook a lot of delicious and healthy food for others since he is limited to what he can eat. He wants to share the love and joy of food in a healthy way. He also likes to play soccer but has stopped playing ever since he had a catheter on. My 6 year old is still deciding whether he should be a doctor or a policeman or a taxi driver. As their mother, I just want them to grow up happy and not have any more pains then their illness has given them.

I wish all my children can have a chance to dream and not be stopped because of rare disease - aHUS.

I remember reading an online article about a mother of two children with aHUS. She lost the older child to this disease. The younger was fortunate because he got to use Eculizumab. After using it, there were no relapses anymore. They can enjoy the quality of life again with that healthy body like doing sports like swimming and traveling. I do not wish to experience losing any of my children to this disease. There is help for this; aHUS. I wish for this help. I wish that he can have a normal life like hanging out with friends. I wish to travel with him. I wish he can enjoy whatever sportswear likes. Please do consider to enlarge the drug usage of Eculizumab for patients with aHUS.

Yours Sincerely,

Tara Wanye Sam