



July 9, 2020

Name: Finn, Conner
DOB: March 29/16
PHN: 182 277 186

To Whom It May Concern,

Conner is a child who has recently been diagnosed with an inherited metabolic disease called X-linked Adrenoleukodystrophy (XALD). This condition affects both the central nervous system and the adrenal glands causing a progressive loss of myelin in the brain and spinal cord as well as adrenocortical insufficiency. The most severe form of the disease, called Childhood Cerebral Adrenoleukodystrophy (CCALD) is a devastating neurodegenerative condition which can affect boys ages 3 to 13. Boys with CCALD have progressive deterioration of cognition, behavior, vision, hearing, and motor function, leading to total disability and death within six months to two years after symptom onset. Bone marrow transplantation is a life-saving treatment for boys who have evidence of brain involvement on MRI but do not yet have significant neurological symptoms or signs of the disease. Gene therapy clinical trials are available in the United States if the child is eligible for the program, and results showing success for this treatment have been published.

Conner's recent brain MRI has shown significant changes, and he therefore has a diagnosis of CCALD. Since Conner is still clinically symptom-free, we have a small window of time in which a bone marrow transplant or gene therapy could be effective. Conner has been offered the opportunity to have a bone marrow transplant within the next two weeks by Drs. Orchard and Lund in Minneapolis, Minnesota. If receiving treatment in Minneapolis, Conner is also eligible for entry into their gene therapy clinical trial should a space become available. Gene therapy would likely be superior to bone marrow transplant as the risks to Conner would be lower, and therefore should be pursued if possible.

It is my clinical opinion that the opportunities offered to Conner through Drs. Orchard and Lund at the Pediatric Blood & Marrow Transplantation Center at the University of Minnesota, Minneapolis would be of great benefit to Conner and could significantly alter his disease course for the better. There is no center in Canada with Minneapolis's level of experience in transplanting children with XALD, a procedure that is associated with up to a 20% mortality rate. **I urge the Saskatchewan Ministry of Health to consider the family's request for coverage of 25% of the cost of the bone marrow transplant to be performed in Minneapolis** so that Conner may have the best opportunity for treatment and outcome. I understand that this 25% would reflect the cost to the province if the bone marrow transplant was to be performed in Canada.

Sincerely,

A handwritten signature in cursive script, appearing to read 'M. Chard'.

Marisa Chard, MD, FRCPC, FCCMG
Metabolic Physician
Saskatchewan Metabolics Program
Saskatoon, SK