Sunday, February 27, 2022

Senate Public Health and Welfare Committee

Kansas State Capitol

SW 8th and SW Van Buren

Topeka, Kansas 66612

Testimony in Support of the Passage of Senate Bill #155

My name is Kirsten Finn and I thank you for allowing me the opportunity to address you today as a proponent for the passage of Senate Bill #155, proposing to increase funding capitation for the Kansas newborn screening program.

I am originally Canadian, but now a proud resident of Overland Park Kansas. I am currently a member of the Newborn Screening Advisory Council and serve on the subcommittee for the initiative to add X-ALD. I also volunteer with organizations such as ALD Alliance, ALD Connect and serve on the Board of Directors of X out ALD to raise funds for research improving treatments for this condition.

I come before you today as the mother of a five-year old boy who was diagnosed by chance with Adrenoleukodystrophy, or ALD. ALD affects one in roughly 17,000 births and is a relentless and progressive neurodegenerative illness, that if left untreated can result in death in six months to two years. ALD destroys the myelin in the brain and boys lose their ability to see, hear, walk and communicate until they fall into a vegetative state and die. Men who develop the adult-onset form of the disease are often misdiagnosed with psychiatric conditions and become homeless or often incarcerated for actions that were simply beyond their control due to undetected cerebral disease. Women with ALD are often misdiagnosed with conditions like MS. All ALD boys develop adrenal insufficiency at some point and this alone can be fatal if left untreated.

The only treatment known to halt the progression of this disease is a blood and marrow transplantation. The window for being eligible for treatment is exceedingly narrow and a later transplant can accelerate rather than halt the disease. Newborn Screening for conditions like ALD is essential and life saving.

My son was diagnosed by chance, shortly after his fourth birthday, at the beginning of the pandemic. We were in Canada at the time and could not get a timely MRI and by the time we did we were devastated to learn he had developed cerebral involvement, the most severe and fatal form of the disease.

No one in Canada could tell us if we were in the window for transplant. In addition, there are no ALD Specialists in Canada and they could not give us any timeline for treatment.

We were denied out of country coverage and were forced to liquidate our retirement savings in order to bring our son to the University of Minnesota Leukodystrophy Center where our son received a life saving bone marrow transplant in August of 2020. Our son has fine motor delays, cerebral visual impairment and auditory processing issues, but these conditions are treatment and he is a very happy kindergarten boy. We are forever indebted to Dr. Troy Lund at the University of Minnesota for saving our son's life. We had the best possible outcome that we could have hoped for give our son's stage of diagnosis

The reason I am so passionate about speaking to you today is that we are the lucky ones. I shudder to think of how close we came to losing our son, or how he might have been condemned to a life of severe and debilitating disability had a transplant been performed too late. If we had the benefit of newborn screening, we would have been able to monitor our son from birth and he could have accessed treatment earlier, thus limiting the disabilities he now faces.

Newborn screening has a far-reaching impact for every positive screen. Siblings, cousins, and adults alike are identified with every positive screen and have the ability to receive life saving and appropriate treatment. They also have the ability to have the benefit of this knowledge in future family planning. This knowledge gained with newborn screening quite simply saves lives and prevents the devastation faced by families who come to a later diagnosis.

I have met so many families from across the US and around the world who received transplants too late. Their children have severe disabilities and cannot see, hear, must be tube fed and have to be trached. These children will never improve and will never return to their former level of function. I have also met many families who have devastatingly come to diagnosis when they are too late for transplant. These families then must take their child home and watch them slowly lose every functional ability they have before they fall into a vegetative state and die.

What I have seen with ALD is true for so many other diseases on the newborn screen panel. These rare diseases often mimic other conditions and the potential for misdiagnosis is exponential. Usually, unless rarely diagnosed by chance like our son, most children are diagnosed too late for any kind of meaningful intervention.

There have been significant advancements made in blood and marrow transplantation and innovative gene therapies for many of these conditions, but these interventions can only be performed with early diagnosis.

Receiving a fatal diagnosis for your child is traumatic. My heart breaks for the families who have lost children and who must every day live with the fact that had screening been in place, they could have saved their child. Families who do receive screening also face trauma as they learn to navigate complex medical conditions and they need immense support as they day after day must face the unknown and the possibility of having to undergo a life saving procedure.

I am very passionate about advocating for newborn screening and in doing what I can for all rare disease families, regardless of their diagnosis. A robust and comprehensive screening program is essential to ensure every baby has the right to life, and to achieving their best possible outcome. I don't ever want to see any family go through what we did or worse.

I stand before you today to implore you to pass Senate Bill 155, so that the Kansas Newborn Screening Program can continue to sustainably provide every Kansas child their best chance in life.

Thank you.

Sincerely,

Kirsten Finn