

March 18, 2019

Chair Salinas, Vice Chairs Nosse and Hayden, members of the committee,

My name is Nikki Monaco, RN, BSN. Thank you all for the opportunity to share our testimony last week. As you can see, I'm starting to learn this process and the proper etiquette! It was truly an honor to speak with you and be able to bring Emmett's story to your attention. You have now heard our personal testimony and I wanted to take a moment to share with you the facts that I have since learned about Krabbe and other diseases we are not currently testing for as a state.

Some statistics you may already know is that it's estimated that 1:100,000 are born with Krabbe. What you may not know is that 90% of cases are early infantile onset and is the most aggressive form that is fatal within the first two years of life, if not caught and treated. This form is usually mistaken as cerebral palsy and goes misdiagnosed until and even after death. The other 10% of cases are either late infantile, juvenile or adult onset. The adult onset is typically mistaken for multiple sclerosis and is often never truly diagnosed. So the fact that Emmett celebrated his 3<sup>rd</sup> birthday literally makes it a one in a million birthday celebration! However, it is heartbreaking because it also means that he had a larger window to be diagnosed and treated compared to the early onset form.

It is vital that this disease is caught early so that parents have the choice to treat their child. There are currently two experts on the disease, Dr Maria Escolar and Dr Joanne Kurtzberg. I know that you have received some testimonies from families who were caught early enough with early infantile onset for treatment to be effective. I hope these have been impactful and show how miraculous these treatments can be! Dr Escolar has been working with Krabbe patients exclusively for the last 20+ years. She diagnosed a child in med school and has not looked back. Currently she is working on gene therapy, which would be a cure, but this would only be offered to those who have been transplanted. She studied under Dr Kurtzberg who has patients that were transplanted and they are in their 20s now. She is currently working on stem cell therapy to reverse the damage to the nerve cells and will be starting human trials within the next year!

Something that we were told in Pittsburgh by Dr Escolar was that we would be the experts when we went home. I have found this to be true! The education among the OB/GYNs, pediatricians, specialists and ER pediatricians is next to nothing! The most I've heard is that it sounds familiar but only because they read about it in a textbook in med school. Therefore, newborn screening is essential to catch these children including those affected by other diseases such as adrenoleukodystrophy (ALD) that affects 1:20,000 babies born. This is another disease with effective treatment and screening available that Oregon does not currently screen!

The relative cost to implement Krabbe disease along with the other lysosomal storage disorder, Niemann-Pick disease, is next to nothing. It costs \$6 to run the test

to screen for one of the six lysosomal storage disorders or for ALL six of them. We are already paying the \$6 per test so it seems only logical to add in Krabbe and Niemann-Pick disease. When looking into the Recommended Uniform Screening Panel (RUSP) that was formed at the Federal level, I learned something about the Public Health Service Act. Under section 2713, it states that ALL non-grandfathered health insurances MUST pay for newborn screens approved by the HRSA at no cost to the insured within one year of being added to RUSP.

My hope is that Oregon can help lead the nation to modernize their screenings of newborns in order to help the maximum number of families possible. Since Oregon is a regional test facility, we can set the example for the other states that send their screenings to us and we can help the families in territories and different tribes to have the best chance of life for their babies! As of right now, we are preventing New Mexico from screening their children. They have passed legislation to screen for Krabbe but are unable to do so since they send their tests to us.

Please support HB 2563 so that we can accomplish this goal. Please help Emmett make a lasting legacy by saving future families from living our horrible reality!

Thank you for your time and consideration.

Sincerely,

Joe, Nikki, Jaxon and Emmett Monaco