MARCUS CENTER FOR CELLULAR CURES
CAROLINAS CORD BLOOD BANK
PEDIATRIC BLOOD & MARROW TRANSPLANT PROGRAM
STEM CELL TRANSPLANT LABORATORY

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RE: HB 2563

To Chair Salinas and members of the House Committee on Health Care:

I am writing in support of House Bill 2563 and the addition of Early Infantile Krabbe Disease and Adrenoleukodystrophy to Oregon's Newborn Screening Blood Spot test. I am a pediatric blood and marrow transplant specialist. I have treated patients with leukodystrophies, leukemias and other blood disorders, many of which are resistant to standard treatments. I am also a researcher focusing on new therapies for children with Krabbe disease, Adrenoleukodystrophy and other leukodystrophies. In over 30 years of treating children with these diseases, I have seen the landscape of testing and treatment change dramatically.

Most babies with early infantile Krabbe disease start showing symptoms around 3-6 months of age, and without treatment they have progressive loss of motor and cognitive functions, develop seizures, spasticity and blindness and die in early childhood. However, with very early detection and treatment in the first month of life with hematopoietic stem cell transplantation, these patients can thrive. I have transplanted over 20 patients in the first month of life and seen life extended to 2 decades or longer and quality of life dramatically increased.

Oregon currently tests for 4 out of 6 Lysosomal Storage Disorders that can be tested for using a simultaneous testing process. Oregon currently screens for Pompe, MPS I, Fabry and Gaucher diseases, and could add Krabbe disease and Adrenoleukodystrophy using only one test for all 6 diseases. Once a newborn is screened and shown to be likely to develop Krabbe, additional testing with the biomarker psychosine at Mayo can rapidly distinguish whether or not the baby has the early infantile form of the disease and needs immediate referral for transplantation. For another leukodystrophies or later onset Krabbe disease, the next step is just monitoring for symptoms during typical well-baby checks during a child's first year of like, then in check-ups every 3-6 months following. This is not an expensive process, especially in a state like Oregon that already prioritizes health care access for children.

Once Krabbe disease, or other leukodystrophy diagnosis is confirmed, effective treatment is available. Hematopoietic Stem Cell Transplantation utilizing unrelated donor umbilical cord blood has been shown to be very effective in treating Lysosomal Storage Diseases like Krabbe Disease. I now have patients who are over 20 years old and thriving. With early testing and treatment, patients have a 90% survival rate, normal cognitive development, and many have normal motor development (30%) or some motor impairment (60%) which is supportable with assistive devices. There is also a lot of promise for future developments in Krabbe and LSD treatment, with improvements being made in transplant outcomes and research going into new potential therapies. OHSU is an excellent example of a research hospital delivering excellent

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bone marrow transplant outcomes, making Oregon already well suited to implement universal Krabbe and LSD screening.

My own research is demonstrating the promising future ahead for Krabbe disease and other Lysosomal Storage Disorders. Using donor cord blood we are working to improve transplants and develop new cell therapies. I have treated 23 babies using donor cord blood and have found it to be safe and effective in improving the motor development of patients.

In conclusion, the State of Oregon is in a great position to expand its Newborn Screening Blood Spot test to include Krabbe Disease and Adrenoleukodystrophy. Oregon already tests for other LSDs which can all be tested for using the same simultaneous test. OHSU has demonstrated its ability to provide great outcomes to patients with these diseases, and with research going into improving therapies, outcomes for children diagnosed with these diseases are more and more optimistic. Expanding Oregon's universal Newborn screening to include Krabbe Disease and Adrenoleukodystrophy will save lives and prevent families from suffering while their child becomes sick without know why.

I urge you to support HB 2563.

Sincerely,

Joanne Kurtzberg, MD
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Professor of Pathology
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