



## **Statement of Maryland Rural Health Association (MRHA)**

*To the Health and Government Operations*

*Chair: Delegate Joseline A. Pena-Melnyk*

*February 5, 2024*

**House Bill 00096: Health - Newborn Screening Program - Krabbe Leukodystrophy**

**POSITION: SUPPORT**

*Chair Pena-Melnyk, Vice Chair Cullison, and members of the committee, the Maryland Rural Health Association (MRHA) is in SUPPORT of House Bill 0096 Health - Newborn Screening Program - Krabbe Leukodystrophy.*

*Krabbe Leukodystrophy is a neurodegenerative disorder that impacts about 1 of every 250,000 infants in the United States. Although rare, the disease is complex and aggressive in nature (Cleveland Clinic, 2022). Caused by a deficiency of an enzyme that helps to preserve myelin (the protective coating on the body's nerves), the nerves and brain are unable to communicate signals properly. Without functional communication between nerves and the brain, in early stages, neurological symptoms such as irritability, difficulty feeding, and failure to thrive will manifest. However, once the disease progresses, blindness, deafness, decreasing mobility and seizures will occur. In most cases, infants will begin to exhibit symptoms around 4-6 months of age. The average life expectancy of infants with krabbe leukodystrophy is around 13 months. Treatment options are very limited, and since there is no cure, treatment is centered around symptom management. One common treatment is to replace impacted cells with healthy one, otherwise known as a hematopoietic stem cell transplant (HSCT). HSCT has proven to delay cognitive and motor deterioration, but only when administered prior to onset of symptoms (Jain & De Jesus, 2023). When considering the aggressive nature and progression of the disorder, the Maryland Rural Health Association supports this bill as it will aid in early diagnosis and proper treatment for affected infants. Newborn screenings for krabbe leukodystrophy will allow the medical team time to properly care for the infant and inform parents on the prognosis of their child as they begin to cope. Despite the absence of a cure for krabbe leukodystrophy, early diagnosis and treatment is one plan of care that can easily be done to improve the quality of life of the infants of Maryland who face the disorder.*

*On behalf of the Maryland Rural Health Association,  
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Cleveland Clinic. (2022). *Krabbe disease (globoid cell leukodystrophy)*.  
<https://my.clevelandclinic.org/health/diseases/6039-krabbe-disease-globoid-cell-leukodystrophy>

Jain, M., & De Jesus, O. (2023). *Krabbe disease*. *National Library of Medicine*. <https://www.ncbi.nlm.nih.gov/books/NBK562315/>