NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

January 30, 2024

The Honorable Ben Hansen
Chairperson, Nebraska Health and Human Services Committee
Nebraska State Capitol Building
1445 K Street, Lincoln, NE 68508

The Honorable Brian Hardin
Vice Chairperson, Nebraska Health and Human Services Committee
Nebraska State Capitol Building
1445 K Street, Lincoln, NE 68508

Dear Chair Hansen and Vice Chair Hardin,

On behalf of the one-in-ten individuals in Nebraska living with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for the opportunity to provide comments on LB 1060. LB 1060, if passed, would provide for an exemption to newborn screening, a critical public health program that saves thousands of lives every year. NORD urges you to oppose LB 1060 and not advance the bill out of the Health and Human Services Committee.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services. We believe that all individuals with a rare disease should have access to high quality, affordable health care that is best suited to meet their medical needs.

Newborn screening is a key public health program with a long history of successful operation in the United States. Screening began in the 1960s when Dr. Robert Guthrie developed a blood test for a condition called phenylketonuria (PKU). PKU is a serious metabolic disorder that causes brain damage if it is not detected and treated very early in life. Children with PKU appear healthy at birth, but they are born without an enzyme necessary to break down certain proteins. As a result, an amino acid called phenylalanine builds up in the body causing permanent damage. Before Dr. Guthrie's blood test, children with PKU weren't diagnosed until after they had irreversible brain damage. The blood test allowed health care providers to detect PKU shortly after birth, enabling earlier treatment and avoiding serious health complications caused by the condition. Now, over 4 million newborns across the country are screened each year for certain rare conditions that, like PKU, can cause permanent disability or death in the absence of early detection and treatment. Babies born with these serious conditions typically appear healthy at birth, so screening is critical to ensure newborns who need it receive early care, treatment, or intervention as early as possible. Over 12,000 babies are found to have a

1779 MASSACHUSETTS AVENUE NW, SUITE 500
WASHINGTON, DC 20036
T 202-588-5700 ▪ F 202-588-5701

7 KENOSIA AVENUE
DANBURY, CT 06810
T 203-744-0100 ▪ F 203-263-9938

1900 CROWN COLONY DRIVE, SUITE 310
QUINCY, MA 02169
T 617-249-7300 ▪ F 617-249-7301

rarediseases.org ▪ orphan@rarediseases.org

serious rare disorder through newborn screening each year. In many cases, these early diagnoses are lifesaving.

Nebraska currently screens for 35 rare conditions. All 35 conditions on Nebraska's screening panel are included on the Recommended Uniform Screening Panel (RUSP). To be added to the RUSP, a condition must meet all of the following criteria: 1) be a serious medical condition; 2) have a clear and well-described case definition; 3) have a screening process that has high analytical validity; 4) have a screening process that is clinically useful; 5) have well-defined treatment protocols; and 6) have data available demonstrating that population-based screening works well to find newborns with the given condition.[1]

Because newborn screening is so important for protecting the health of babies affected by the conditions on the screening panel, newborn screening is mandatory in Nebraska. Parents who forgo newborn screening risk missing critical information about their child's health, and children with these rare conditions can suffer preventable, irreversible health complications as a result. In its current form, LB 1060 allows parents to opt out of newborn screening but does nothing to ensure parents understand the lifesaving purpose of the screening and the risks of opting out. NORD strongly advocates that all newborns should be screened for these conditions and parents should not opt out of screening, but any opt out provision must, at the bare minimum, include a requirement that parents are educated about the importance of newborn screening before being allowed to decline to have their newborn participate. This bill puts children at risk of suffering serious and permanent preventable health complications, and we urge the Committee to oppose LB1060 as drafted.

Once again, on behalf of the Nebraska rare disease community, we thank you for the opportunity to provide comments on LB 1060. For any questions, please feel free to contact Allison Herrity at aherrity@rarediseases.org.

Sincerely

Lindsey Viscarra
State Policy Manager, Western Region
National Organization for Rare Disorders

Allison Herrity
Policy Analyst
National Organization for Rare Disorders