NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

March 12, 2024

The Honorable Bryan Terry
Chair, House Committee on Health
425 Rep. John Lewis Way N.
Suite 646 Cordell Hull Bldg.
Nashville, TN 37243

The Honorable Tom Leatherwood
Vice-Chair, House Committee on Health
425 Rep. John Lewis Way N.
Suite 514 Cordell Hull Bldg.
Nashville, TN 37243

Re: NORD Supports HB1973 An Act Relative to Newborn Screening

Dear Chair Terry, Vice-Chair Leatherwood, and Members of the House Committee on Health,

On behalf of the one-in-ten individuals in Tennessee living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for the opportunity to provide comments on HB1973 (Hawk). If passed, HB1973 would require Tennessee to add conditions that appear on the Recommended Uniform Screening Panel (RUSP) to the state newborn screening panel within 36 months of their addition to the RUSP. The Senate companion bill, SB1791 (Massey), passed with bipartisan unanimous support on March 4th. NORD urges you to support HB1973 and advance the bill through the Health Committee to the full Tennessee House of Representatives.

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, improving the health of many children through timely diagnosis and treatment. 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 suffered 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 a number of 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 serious rare disorder through newborn screening each year. In many cases, these early diagnoses are lifesaving and as science progresses, an increasing number of conditions are being added to the screening panel, helping to save additional lives.

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Newborn screening programs are operated at the state level, and the specific conditions on each state's newborn screening panel varies across the country. To guide state newborn screening programs, the Health Services and Resources Administration's Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) maintains a list of conditions recommended for screening called the Recommended Uniform Screening Panel. 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] Tennessee currently screens for 35 of the 37 conditions on the RUSP. For conditions to be added to the state panel it takes between six months to one year and requires approval by the State Advisory Committee and Commissioner of Health

By not screening newborns for all conditions on the RUSP, the state risks missing critical information about newborn Tennesseans' health, and children with conditions that are not on the state panel can suffer preventable, irreversible health complications as a result. HB1973 requires the state to add conditions that appear on the RUSP to the state newborn screening panel within 36 months of the condition's addition to the RUSP. If the state is unable to meet this deadline, the bill requires the State Board of Health to provide a written explanation for the delay to the State Health Officer. NORD supports the efficient addition of RUSP conditions to state newborn screening panels and the process laid out in this bill. We urge the Committee to support this legislation and swiftly advance it out of Committee.

Once again, on behalf of the Tennessee rare disease community, we thank you for the opportunity to share NORD's support for HB1973. For any questions, please feel free to contact Allison Herrity at aherrity@rarediseases.org or Carolyn Sheridan at csheridan@rarediseases.org.

Sincerely,

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CC:
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