



Make today a breakthrough.

**Written Testimony of**  
**Kenneth Hobby, President, Cure SMA**  
**for the**  
**Kansas Senate Public Health and Welfare Committee**  
**February 24, 2020 Committee Hearing**  
**to consider**  
**Senate Bill 364 to Expand and Fund**  
**Kansas Newborn Screening Services**

Chairman Suellentrop and Members of the Committee,

My name is Kenneth Hobby and I am President of Cure SMA, the leading national organization dedicated to funding research, promoting access to treatments and care, and finding a cure for Spinal Muscular Atrophy or SMA.

Thank you for holding a hearing on Senate Bill 364 (SB 364) to expand and fund the Kansas Newborn Screening Program. On behalf of Kansas individuals and families impacted by SMA, **Cure SMA fully supports SB 364 which would, among other things, make available the resources needed to add federally-recommended conditions to the Kansas Newborn Screening Program.**

SMA is a progressive neurodegenerative disease caused by a mutation in the survival motor neuron gene 1 or SMN1. In a healthy person, this gene produces a protein that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, which can significantly impact an individual's ability to walk, swallow, and—in the most severe cases—breathe. SMA affects 1 in 11,000 births in the United States each year, and approximately 1 in 50 Americans is a genetic carrier. **In Kansas**, 3 babies with SMA are born each year, adding to the estimated 115 state residents already living with SMA, according to Cure SMA estimates. In addition, about 58,000 Kansas residents are carriers of the SMA genetic mutation.<sup>i</sup>

In July 2018, U.S. Health and Human Services Alex Azar approved the Advisory Committee on Heritable Disorders in Newborns and Children recommendation to add SMA to the Recommended Uniform Screening Panel (RUSP).<sup>ii</sup> However, each state must act

individually to adopt and implement a RUSP-recommended condition to the state's newborn screening program.

**Cure SMA was grateful that the Kansas Department of Health and Environment added SMA to the state's Newborn Screening Program and started screening Kansas newborns for SMA on February 1, 2020.** As a result, Kansas families with children who are diagnosed with SMA through a newborn screening test can make timely decisions on care and treatment. There are two FDA-approved SMA treatments (with a third in FDA priority review) that make it possible for individuals with SMA to achieve unprecedented developmental milestones. These treatments are most effective when delivered early, before nerve and muscle strength are lost forever.

SB 364 would authorize the Kansas Secretary of Health and Environment to add other RUSP-approved conditions to the Kansas Newborn Screening Program. In addition, the legislation would make minor changes to existing statute and transfer resources from the Kansas Newborn Screening Fund necessary to implement these RUSP-approved conditions. **Cure SMA understands the impact an early diagnosis can have on the ability to treat and care for a rare condition, such as SMA, and we respectfully urge you to adopt this legislation.**

Thank you for considering the views of Cure SMA and Kansas individuals and families impacted by SMA. Please do not hesitate to contact Cure SMA if you have questions or need more information.

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<sup>i</sup> Kansas SMA Fact Sheet, Cure SMA, 2020: [https://www.curesma.org/wp-content/uploads/2020/02/SMA-State-Fact-Sheet\\_2020\\_KS\\_v4.pdf](https://www.curesma.org/wp-content/uploads/2020/02/SMA-State-Fact-Sheet_2020_KS_v4.pdf)

<sup>ii</sup> <https://www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/reports-recommendations/final-sign-azar-response-sma.pdf>