



Make today a breakthrough.

December 4, 2017

Chairman Stephen A. Huffman
Committee on Health
77 S. High St
13th Floor
Columbus, OH 43215

Rnk. Member Nickie Antonio
Committee on Health
77 S. High Street
10th Floor
Columbus, OH 43215

RE: Letter of support and testimony for HB 397-- A bill to amend section 3701.501 of the Revised Code to include spinal muscular atrophy as an additional disorder to be screened for under the Newborn Screening Program

Dear Representatives Huffman and Antonio:

Thank you for the opportunity to submit this letter from Cure SMA in lieu of testimony. On behalf of the 10,000 people living with spinal muscular atrophy (SMA), including the estimated 410 Ohioans living with SMA and the estimated 215,063 Ohio carriers, Cure SMA appreciates the opportunity to communicate our support for HB 397 a bill to screen newborns for spinal muscular atrophy sponsored by Representatives Butler and Boggs.

Cure SMA, is the largest organization in the United States dedicated to the treatment and cure of spinal muscular atrophy (SMA). Cure SMA, in its efforts to fund research activities for SMA and support families affected by the disease, works with 12,000 households in the SMA community, as well as with researchers and health care providers, and with a network of more than 115,000 additional supporters. These stakeholders represent all 50 states as well as dozens of countries. In addition, Cure SMA has funded approximately \$65 million in research. This type of private sector



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investment coupled with a robust federal investment at NIH, CDC, and HRSA helps to facilitate important breakthroughs in screening, diagnosis, treatment, and care.

SMA is the leading genetic cause of death for infants under two years of age. It is an autosomal recessive neuromuscular disease that affects approximately 1 in 11,000 live births in the U.S., and an estimated 1 in 50 Americans is a genetic carrier. SMA is caused by a mutation in the gene Survival Motor Neuron 1 (*SMN1*). In a healthy person, this gene produces a protein that is critical to the function of the nerves that control muscles. Without this protein, those nerve cells cannot properly function and eventually die, leading to debilitating and often fatal muscle weakness robbing patients of their ability to walk, eat, or even breathe. However, SMA does not affect a person's ability to think, learn, and build relationships with others.

The December 23, 2016 approval of SPINRAZA™, the first-ever FDA-approved treatment for the disease provided the opportunity for Cure SMA and members of the SMA Newborn Screening Coalition, to submit an application (final determination anticipated as soon as February of 2018) nominating SMA to be added as a condition on the Recommended Uniform Screening Panel (RUSP). The RUSP is a list of disorders that are screened at birth and recommended by the Secretary for states to screen as part of their state universal NBS programs as outlined in federal law under the Newborn Screening Saves Lives Act of 2014 (P.L. 113-240). In urging the legislature to pass HB 397 we note that federal law does not preempt states from adding SMA to screening panels and we applaud you for recognizing the critical importance of this proposal.

In closing, time has a clear and profound adverse effect on the outcome for SMA patients. Just a few weeks can mean the difference between life



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and death for an infant with SMA type I, where the median survival is between eight and 11 months in recent natural history studies. Treatment for SMA is most effective when it can begin before symptoms appear, making NBS a vital predicate for the most successful treatment of SMA. Now that we have one treatment available, several other treatments in clinical trials, Cure SMA and our families across the country have begun this outreach to states to add SMA to their panels. We are hopeful that Ohio will pass this legislation and join the several other states considering SMA for their newborn screening panels.

If you have any further questions of Cure SMA or need further assistance please don't hesitate to contact Maria Spencer, our Vice President of Policy and Advocacy at 847.709.6341 or via email maria.spencer@gmail.com.

Sincerely,

A handwritten signature in black ink, appearing to read "K. A. Hobby", enclosed in a thin black rectangular border.

Kenneth Hobby, President
Cure SMA

Enclosure

cc. Representative Kristen Boggs
Representative Jim Butler