Make today a breakthrough.

July 11, 2024

The Honorable Michael T. McCaul
The Honorable Anna G. Eshoo
The Honorable Gus M. Bilirakis
The Honorable Nanette Diaz Barragan
The Honorable Michael C. Burgess
The Honorable Lori Trahan
U.S. House of Representatives
Washington, DC 20515

The Honorable Robert P. Casey, Jr.
The Honorable Markwayne Mullin
The Honorable Sherrod Brown
The Honorable Susan M. Collins
U.S. Senate
Washington, DC 20510

Dear Senators and Representatives,

As the leading national organization that represents individuals with a rare disease known as spinal muscular atrophy (SMA), **Cure SMA is pleased to support the Creating Hope Reauthorization Act of 2024** (H.R. 7384/ S. 4583). Your bipartisan legislation would extend the rare pediatric disease priority review voucher (PRV) program, a key tool that has contributed to treatment discoveries for SMA.

SMA is a neuromuscular disease that causes debilitating muscle loss and significantly impairs a person's ability to swallow, walk, and perform other ordinary functions. When the PRV program was established in 2012, SMA was considered the leading genetic cause of infant death. Babies born with SMA Type 1, the most common form of the disease, often died before reaching their second birthday. Those who survived required aggressive care from a multi-disciplinary team of health professionals and high-cost interventions, including permanent ventilation, feeding tubes, and around-the-clock nursing. Researchers had identified the cause of the disease (a faulty survival motor neuron gene), but no treatments were approved or on the immediate horizon.

Today, thanks to rare disease investments and policies such as PRV, there are now three U.S. Food and Drug Administration-approved treatments that are helping to slow or stop future progression. SMA is no longer considered the leading genetic cause of infant death. The SMA mortality rate has decreased by one-third and hospitalizations and reliance on specialized care and equipment are also down. However, there still remains significant unmet needs in the SMA community, especially for children and adults with SMA who had already lost substantial muscle strength and motor function ability before treatment discoveries. Additional research and development are needed to meet this need and to find a cure for the disease.

The Creating Hope Reauthorization Act of 2024 would help address SMA needs and incentize development for other rare diseases by extending the PRV program through fiscal year 2030. The legislation would help maintain our country's commitment and leadership in finding cures for debilitating diseases, such as SMA, and meet the everyday living needs of rare disease patients. *"Research studies necessary to find the cure must be*

continued. Their success will not only save SMA babies lives, they will also correct numerous disability problems. Human genetics affect everyone. Knowing and fixing defects helps everyone," said a **grandparent of a child with SMA**. Another **Cure SMA supporter** said, *"The more research given to this disease, the more hope our children have to live longer, happier lives."*

Cure SMA and the SMA community look forward to working with you to extend this important drug development tool that can provide further hope and results for individuals with SMA and other rare diseases. Thank you again for your support of individuals with SMA and their families. Your staff can reach out to Cure SMA by contacting Maynard Friesz, Cure SMA Vice President for Policy and Advocacy, at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President, Policy
Cure SMA