Make today a breakthrough.

September 25, 2024

Dear Senator,

As the leading national organization that represents individuals with a rare disease known as spinal muscular atrophy (SMA), **Cure SMA respectfully asks that you support the Creating Hope Reauthorization Act of 2024 (S. 4585) during the Health, Education, Labor, and Pensions (HELP) Committee markup**. The 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 SMA 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 incentivize 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."*

The PRV provision is set to expire on September 30. Cure SMA and the SMA community are grateful that a temporary extension (through December 20th) is included in the short-term funding measure (Continuing Resolution) currently being considered in Congress. However,

a long-term PRV extension, as proposed in the Creating Hope Reauthorization, is required to continue the progress our country has made in addressing the healthcare needs of individuals with rare diseases, such as SMA. Your support for the Creating Hope Reauthorization will help ensure that this critical provision is extended through 2030. **For these reasons, we ask that you vote YES to advance the Creating Hope Reauthorization Act.**

Thank you for considering the views of Cure SMA and the individuals with SMA and their families that we represent. 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,

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