



Make today a breakthrough.

May 16, 2024

Dear Representative:

As the leading national organization that represents individuals with a rare disease known as spinal muscular atrophy (SMA), **Cure SMA is pleased to lend our support for bipartisan legislation being considered during today's Energy and Commerce Health Subcommittee markup.** Cure SMA supports the Think Differently Database Act (H.R. 670), if amended to expand the definition of disability, the Creating Hope Reauthorization Act of 2024 (H.R. 7384), and the Accelerating Kids' Access to Care Act (H.R. 4758).

SMA is a neuromuscular 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, taking away an individual's ability to walk, eat, and breathe. SMA impacts 1 in 11,000 births in the United States and approximately 1 in 50 people is a genetic carrier for SMA.

The **Think Differently Database Act** would create a one-stop online source of information about federal, state, and local disability programs and services. As drafted, the legislation would only benefit individuals with intellectual disabilities. Cure SMA supports an amendment to the legislation to expand the definition of disability to include physical and other disabilities. Individuals with SMA struggle to find information about available disability programs and services that could help maintain their health and independence. In our 2024 caregiving report, Cure SMA learned that [47 percent](#) of individuals with SMA and their families struggled to find information about paid caregiving options in their state. *"I couldn't find information directly online about the waiver program that I use. I only found out about it through my counselor at the Division of Rehabilitative Services,"* said a **woman with SMA** featured in our report. Cure SMA applauds the legislation's goal of creating a comprehensive, searchable online clearinghouse of disability information, services, and programs. However, Cure SMA and the SMA community respectfully ask that the Think Differently Database Act be approved with an amendment to expand the scope to include individuals with all disabilities.

The **Creating Hope Reauthorization Act of 2024** would reauthorize the U.S. Food and Drug Administration's rare pediatric disease priority review voucher (PRV) program that incentivizes the development of rare disease treatments. Individuals with SMA have benefited from the PRV program and other policies that have spurred research and development into SMA. Before 2012, when PRV was first authorized, 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. There were no SMA treatments and few candidates in the SMA research pipeline. Today, thanks in part to PRV and other policies, there are three FDA-approved SMA treatments (and numerous others in the research pipeline) that have helped to dramatically reduce the mortality rate and improve the lives of children and adults with SMA. Cure SMA and the SMA community support the Creating Hope Reauthorization Act to extend the PRV program.



The **Accelerating Kids' Access to Care Act** would make it easier for children with SMA and other medically complex conditions to access the specialized care they need, even if that care is in another state. The standard care team for children and adults with SMA may include pediatric neurologists, pulmonologists, and other neuromuscular disease specialists with expert knowledge about SMA. For some individuals with SMA, the specialized care they need is only available across state lines. The current process for out-of-state healthcare providers to enroll in a Medicaid program in a different state is burdensome and time-consuming, which could delay access to life-saving care. Cure SMA and the SMA community support the Accelerating Kids' Access to Care Act to help streamline the out-of-state provider Medicaid enrollment process to ensure that children with SMA and others receive timely access to the specialized care and services they need.

Thank you for considering the views of the SMA community. Please do not hesitate to contact Cure SMA if you have any questions. Your staff can contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202-871-8004 or [maynard.friesz@curesma.org](mailto:maynard.friesz@curesma.org).

Sincerely,

A handwritten signature in black ink that reads "K. A. Hobby".

Kenneth Hobby  
President

A handwritten signature in black ink that reads "Maynard Friesz".

Maynard Friesz  
Vice President of Policy