



Make today a breakthrough.

December 1, 2021

The Honorable Diana DeGette
United States House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Fred Upton
United States House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Upton:

As the leading national organization that supports and advocates for individuals with spinal muscular atrophy (SMA) and their families, **Cure SMA is pleased to support your bipartisan 21st Century Cures 2.0 Act (H.R. 6000) to continue the acceleration of the discovery, development, and delivery of cures and treatments for disease such as SMA.**

SMA is a genetic 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 die, impacting an individual's ability to crawl, walk, eat, and breathe. If both parents are SMA carriers, every child they have together has a 25% chance of being diagnosed with SMA, regardless of race, ethnicity, and gender. SMA impacts 1 in 11,000 births in the United States.

The SMA community has experienced a decade of scientific discoveries and treatment approvals thanks in part to innovative public policies that encourage research and development into rare diseases. In the early 2000s, only a couple of potential SMA drugs were in the beginning preclinical discovery stage. Today, we have three U.S. Food and Drug Administration (FDA)-approved treatments for SMA and 18 additional programs in development, including five in clinical trials. The current SMA treatments came to market using a pediatric priority review voucher established by Congress to provide fast-track consideration at the FDA for promising treatments for rare pediatric diseases such as SMA. This important program was extended through your earlier 21st Century Cures Act.

The three FDA-approved SMA treatments have had a transformative impact on the disease. However, unmet needs remain in the SMA community, especially for adults with SMA, who identified *gaining muscle strength, achieving new motor function, improving daily functioning, and reducing fatigue* among their top needs. **Additional SMA research investments are required to meet these pressing SMA community needs**, which is why Cure SMA applauds your efforts to continue the focus on and investment in new treatments and cures through the proposed Advanced Research Project Agency for Health (ARPA-H) and independent research institutions.

The 21st Century Cures 2.0 Act will maintain progress and discovery in SMA and other rare diseases through key provisions supported by Cure SMA. Drug development and coverage must reflect patient needs and real-world experiences, especially related to outcomes



that really matter and make a difference in their everyday lives. For example, an increase in strength that was recorded as minimal has provided exponential gains for an adult with SMA. He described how his current SMA treatment allows him to lift his elbows off the armrest of his motorized wheelchair by 2 inches, which has allowed him to eat independently, greatly improving his abilities, independent living, and quality of life. Your legislation recognizes the importance of patient-reported data and real-world experience.

In addition, Cure SMA supports the legislation's emphasis on telehealth, cell and gene therapies, digital health technologies, caregiver training, and diversity of clinical trials. Finally, we appreciate your inclusion of language to prohibit the use of geographic tracking features and biometrics in electronic visit verification (EVV) systems. Individuals with SMA and others who utilize personal care attendants have raised privacy concerns ever since the EVV requirement was included in the original Cures Act. The EVV section of Cures 2.0 makes clear that EVV systems are not allowed to capture location or other personal data as the individual and their caregiver move throughout the community for appointments or other activities.

Thank you for your leadership and efforts to continue research innovation and drug breakthroughs to meet the needs of individuals with SMA and their families. Cure SMA strongly supports the 21st Century Cures 2.0 Act (H.R. 6000) and we stand ready to assist you and your teams in securing passage in the 117th Congress. For more information, your staff can contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

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

Kenneth Hobby
President
Cure SMA

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

Maynard Friesz
Vice President of Policy & Advocacy
Cure SMA