



Make today a breakthrough.

January 31, 2023

The Honorable Cathy McMorris Rodgers
Chair, Energy and Commerce Committee
United States House of Representatives
Washington DC 20515

Dear Chair McMorris Rodgers,

Cure SMA, which represents individuals with a neuromuscular disease known as spinal muscular atrophy (SMA), **is pleased to support the Protecting Health Care for All Patients Act to prevent health care discrimination on the basis of disability.** Your legislation would prohibit Medicaid and other federal health programs from using discriminatory measurements, such as quality-adjusted life years (QALYs), when determining coverage or payment for life-saving treatments, services, and devices.

SMA is a progressive neurodegenerative disease that is caused by a mutation in the survival motor neuron gene 1 (SMN1) that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, leading to debilitating and often fatal weakness in muscles used for breathing, crawling, walking, head and neck control, and swallowing. In contrast to the progressive motor weakness, individuals with SMA have normal cognition and excel in school and the workforce and are contributing greatly to society.

Access to healthcare, including treatments, caregiving, and medical devices, is vitally important to children and adults with SMA. Three U.S. Food and Drug Administration-approved treatments exist that are targeting the underlying genetics of SMA and stopping or slowing future degeneration. Medical devices, such as ventilators (BiPAP machines) and power wheelchairs, are making it possible for children and adults with SMA to live independently and navigate their communities. And many individuals with SMA rely on personal care attendants (PCAs) and other in-home aides to assist with everyday living activities, such as bathroom transfers, meal preparation, and getting out of bed and dressed in the morning to attend work or school. Despite research and real world evidence that concludes timely and full access to these treatments, health care services, and devices improves the health and well-being of individuals with SMA, many report that they struggle to access these essential services. Individuals with SMA and their families shared in a 2022 report that *“battling insurance and state Medicaid programs is frustrating and requires time and energy.”* Cure SMA’s Community Survey found that a majority of adults with SMA (56%) and parents of children with SMA (61%) have had to appeal an insurance denial related to SMA treatment coverage, the majority of whom receive coverage through Medicaid. *“Please advocate on behalf of the SMA community to ensure that we are able to continue receiving treatment and care,”* an adult with SMA shared as a concern during a recent health-related survey.



The use of QALYs and other measurement tools that devalue a person with a disability can contribute toward negative coverage determinations. The National Council on Disability, in its 2019 report to Congress, found that QALY measurements, which *“may have a negative impact on the health and welfare of people with disabilities,”* are increasingly used in reimbursement and drug pricing decisions. ICER, for example, regularly uses QALYs to determine what it considers fair market value of drugs, including life-saving treatments for ultra-rare diseases such as SMA. In addition, during the COVID-19 pandemic, several states released rationing of care guidelines that excluded or deprioritized people with SMA and other disabilities from accessing ventilators and other medical care based solely on their disabilities. *“People with SMA need equal treatment in medical triage settings,”* a SMA community member shared during the height of the pandemic.

The Protecting Health Care for All Patients Act would stop this discriminatory practice against people with SMA and other disabilities by prohibiting the use of QALYs and similar measurements under any federal health care program. We stand ready to assist you and your team in securing passage of this important legislation during the 118th 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