



Make today a breakthrough.

February 3, 2022

The Honorable Patty Murray  
Chair  
Health, Education, Labor & Pensions Committee  
United States Senate  
Washington, DC 20510

The Honorable Richard Burr  
Ranking Member  
Health, Education, Labor & Pensions Committee  
United States Senate  
Washington, DC 20510

Dear Chair Murray and Ranking Member Burr:

As the leading national organization that supports and advocates for individuals with spinal muscular atrophy (SMA) and their families, **Cure SMA appreciates the opportunity to review and comment on your discussion draft of the Prepare for and Respond to Existing Viruses, Emerging New Threats, and Pandemics Act (PREVENT Pandemics Act).**

SMA is a progressive neurodegenerative disease that robs individuals of physical strength, taking away their ability to walk, eat, and breathe. SMA impacts 1 in 11,000 births in the United States and 1 in 50 Americans is a SMA carrier. 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.

Neuromuscular and respiratory muscle weakness associated with SMA can be exacerbated by viral illness, which puts people with SMA at higher risk of serious illness from infectious diseases such as COVID-19 or the seasonal flu. The COVID-19 pandemic also exposed other vulnerabilities for the SMA community from isolation and healthcare discrimination to access to mental health supports and caregiving services. Our country must be better prepared in a future pandemic to respond to the unique needs of at-risk populations, including individuals with SMA.

**Cure SMA applauds your bipartisan leadership and efforts to help prepare our country for future public health emergencies.** We are pleased to highlight and comment on the following provisions (listed as they appear in the bill) that are especially impactful to the SMA community.

- **SEC 101—COMPREHENSIVE REVIEW OF THE COVID-19 RESPONSE:** Cure SMA supports the establishment of a national task force to examine challenges, identify best practices, and review policy decisions associated with the COVID-19 pandemic. Lessons learned from this comprehensive examination will help to inform local, state, and federal leaders as they plan and implement strategies for future pandemics. Under the legislation, task force members should be “highly qualified citizens” and be selected for having expertise in one or more areas, including “health disparities and at-risk populations.” **Cure SMA asks that you more clearly require that at least one of the task force members has demonstrated expertise and experience in health disparities and at-risk populations given the outsized impact COVID-19 has had on at-risk and minority populations, including individuals with SMA.**

- **SEC.112—SUPPORTING ACCESS TO MENTAL HEALTH SERVICES DURING PUBLIC HEALTH EMERGENCIES:** Cure SMA appreciates that the legislation emphasizes the need for enhanced mental health and substance use disorder services during public health emergencies. Extended periods of isolation and concerns over rationing of care and hospitalizations due to underlying health conditions associated with SMA made the COVID-19 pandemic especially stressful for the SMA community. Adults with SMA and families with children with SMA highlighted their challenges in surveys Cure SMA conducted throughout the pandemic. An adult with SMA wrote, *“Mental health is my biggest problem right now (depressed so hard).”* A parent of a child with SMA shared that *“I am worried about everyone’s mental health and children’s mental health due to not having social activities.”* Another survey respondent said, *“This year has been mentally and emotionally hard on all of us to some degree and there is a strong need to connect in deep ways with one another.”*

Cure SMA responded to that need by launching a [virtual therapy program](#) where individuals with SMA could connect directly with a licensed therapist to help them navigate these challenging and uncertain times. In addition, we organized numerous virtual social opportunities to give individuals with SMA—many of whom were essentially cut off from their normal routines for up to 2 years—to connect with others in the SMA community. While these efforts were impactful and represented some of Cure SMA’s most widely attended activities, the federal government must do everything it can to help support increased and continued access to mental health services. **Cure SMA supports the strategic plan and advisory council provisions included in the discussion draft. In addition, we encourage you to authorize emergency mental health grants to ensure that community providers have access to resources during future public health emergencies to meet the unique mental health needs of all Americans, especially at-risk populations, such as individuals with SMA and their families.**

- **SEC. 201—ADDRESSING SOCIAL DETERMINANTS OF HEALTH AND IMPROVING HEALTH OUTCOMES:** Cure SMA appreciates that the discussion draft proposes a new grant program focused on addressing health disparities among underserved populations. **Cure SMA asks that individuals with rare diseases, such as SMA, be included in the definition of underserved populations, especially those individuals living in rural areas and from racial and ethnic minority populations.**
- **SUBTITLE C—REVITALIZING THE PUBLIC HEALTH WORKFORCE:** Cure SMA is pleased that the discussion draft includes several major provisions under Subtitle C to strengthen the public health system and workforce, which experienced enormous strains throughout the COVID-19 pandemic. For example, several state health departments reported to Cure SMA delays in their plans to implement newborn screening of SMA, as recommended by the federal government, due to staffing shortages or workplace operational challenges. The COVID-19 pandemic highlighted the vulnerabilities of a historically under-funded public health system. Cure SMA is pleased that the discussion draft focuses on this nationwide challenge.

Cure SMA supports the additions proposed to the Public Health Service Act (Sec. 221) to further clarify who qualifies under the Public Health Workforce Loan Repayment Program. The addition of individuals who work in or are studying laboratory sciences will help to address the national shortage of these essential workers. In addition, the GAO public health workforce study will help to identify public health workforce gaps and recommendations for recruitment and retention. Cure SMA also supports the new grant program (Sec. 222) to better meet the needs of underserved communities by increasing workforce capacity. Cure SMA found that access to personal care attendants (PCAs) and other in-home health workers was significantly limited during the COVID-19 pandemic due to various factors. **Cure SMA asks that PCAs and other in-home workers are included in the “community health worker” definition within the Sec. 222 community health workers program given their role in providing and promoting individual and community health and independent living. This addition would also help meet the program’s goal of addressing social determinants of health and eliminating health disparities.**

- **SEC 502—MODERNIZING CLINICAL TRIALS:** Research into treatments and a cure for SMA is a primary focus for our organization. Currently, 3 FDA-approved treatments exist for SMA with others in various stages of drug development, including clinical trials. Clinical trial recruitment can be challenging for rare disease communities, such as SMA, especially related to clinical trial diversity, a key Cure SMA priority. **Cure SMA is pleased that the discussion draft will help address recruitment, diversity, and retention challenges through the use of digital health technologies, decentralization, and other recommendations for reducing the barriers to clinical trial participation.**
- **SEC. 505—FACILITATING THE USE OF REAL WORLD EVIDENCE:** Cure SMA has worked with other national organizations and coalitions in elevating the use of patient-reported data and real world evidence in all aspects of drug and medical device research, development, and deployment. Cure SMA is developing a Clinical Care Network of research and treatment sites aimed at improving patient care and increasing treatment access through real world data and evidence collected from individuals with SMA and their families. **Cure SMA supports the new authority provided in the discussion draft for the Secretary of Health and Human Services (HHS) to issue or revise existing guidance for using real-world data and evidence related to emergency-use authorizations of drugs and medical devices.**
- **SEC. 516—PREVENTING MEDICAL DEVICE SHORTAGES:** Given weak or underdeveloped lung capacity associated with SMA, some individuals with SMA utilize home ventilators for breathing support. The ventilator shortage and threat of rationing of care policies at the beginning of the COVID-19 public health emergency were particularly troubling for those individuals and their families who were already dealing with other pandemic-related challenges. Cure SMA supports efforts, as proposed in the discussion draft, for the HHS Secretary to mitigate or prevent a shortage of medical devices, such as





ventilators, that are essential for both home and hospital environments. **Cure SMA asks that medical devices developed through these new authorities and pilot programs ensure priority access to high-risk populations, including individuals with SMA.**

The COVID-19 pandemic has been challenging for all Americans, including individuals with SMA and their families. One individual with SMA summed it up in the simplest terms: *“it’s just really difficult right now.”* The provisions proposed in your PREVENT Pandemics Act represents an important step forward in mitigating current challenges so that our nation is better prepared for the next pandemic. Thank you for the opportunity to provide comment on the PREVENT Pandemics Act discussion draft. For more information, your staff can contact Maynard Friesz, Vice President for Advocacy at Cure SMA, at [maynard.friesz@curesma.org](mailto:maynard.friesz@curesma.org) or 202-871-8004.

Sincerely,

  
Kenneth Hobby  
President

  
Mary Schroth, M.D.  
Chief Medical Officer

  
Maynard Friesz  
Vice President of Advocacy