Make today a breakthrough.

May 9, 2022

The Honorable Frank Pallone
Chairman
Energy and Commerce Committee
United States House of Representatives
Washington, DC 20515

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

The Honorable Anna G. Eshoo
Chairwoman
Energy and Commerce Health Subcommittee
United States House of Representatives
Washington, DC 20515

The Honorable Brett Guthrie
Ranking Member
Energy and Commerce Health Subcommittee
United States House of Representatives
Washington, DC 20515

Dear Representatives Pallone, Eshoo, McMorris Rodgers, and Guthrie:

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 Food and Drug Administration Amendments of 2022 (FDA 2022) legislation** that extends and improves key FDA authorities, including programs aimed at accelerating the research, development, and approval of therapies and devices to treat rare diseases 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.

Past Congressional policies and investments have helped to spur treatment discoveries and approvals that have improved the lives of children and adults with SMA and their families. Current SMA treatments can slow or stop future degeneration, helping individuals with SMA to maintain motor function and reduce future healthcare needs. Real world evidence shows that these developmental gains are sustained over time, leading to greater quality of life and independence for individuals with SMA. While current treatments are helping to change the course of the disease, they do not represent a cure, nor do they reverse nerve damage associated with SMA. There remains significant unmet need throughout the SMA community, regardless of age, disease stage, or treatment utilization. That is why Cure SMA is focused on providing federal agencies, including the FDA and the National Institutes of Health, with the resources and the authority they need to expand research, development, and approval of new therapies, devices, and, eventually, a cure for SMA.

The FDA 2022 legislation represents another legislative step forward in helping our nation's public health regulatory agency foster product innovation and advance public safety. The legislation's extension of the user fee programs will ensure the FDA can hire and retain qualified staff to carry out its important public health mission. The legislation also includes important provisions

related to improving clinical trial diversity, including a new diversity action plan requirement, and measures aimed at preparing for future health emergencies, including the advanced manufacturing designation pilot to maintain the supply of health products and an advisory committee focused on pandemic preparedness and response related to medical devices.

Cure SMA especially appreciates the legislation's provisions aimed at addressing the needs of individuals with SMA and other rare diseases and conditions. Your legislation recognizes that individuals with rare diseases and their families are key stakeholders and should be included throughout the drug development and approval process. Section 703 directs the Department of Health and Human Services (HHS) to hold public meetings and a public comment period to collect ideas for increasing and improving engagement with rare disease patients and organizations and requires a report that summarizes FDA best practices around rare disease development and an independent study to assess FDA's rare disease outreach activities. Cure SMA has appreciated the FDA's past and ongoing engagement with the SMA community to understand the disease, efficacy of current treatments, and the urgency for additional treatments to meet unmet needs.

Finally, **Cure SMA welcomes the focus on real world evidence and other patient reported data to help inform regulators about patient need and impact**. Section 805 requires HHS to issue or update guidance on the use of real world data and real world evidence to support regulatory decision making. Section 804 directs HHS to conduct a pilot program and host public workshops related to the development of endpoints for rare diseases, including the use of real world evidence and data to support the validation of endpoints. Cure SMA collects real world data through its annual community survey and its patient registries to ensure key stakeholders, including the FDA, fully understand treatment impact and outcomes that matter most to patients in their everyday lives. Your legislation recognizes the value of this data and evidence.

Thank you for your leadership on this legislation and your efforts to raise awareness of and create solutions for individuals with SMA and other rare diseases. Cure SMA and our supporters across the country look forward to working with you and your staff to secure passage of these important provisions. 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,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President of Policy & Advocacy
Cure SMA