Make today a breakthrough.

February 28, 2022

The Honorable Roger Wicker
U.S. Senate
555 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Amy Klobuchar
U.S. Senate
425 Dirksen Senate Building
Washington, DC 20510

The Honorable Doris Matsui
House of Representatives
2311 Rayburn House Office Building
Washington, DC 20515

The Honorable Brad Wenstrup
House of Representatives
2419 Rayburn House Office Building
Washington, DC 20515

Dear Senators Wicker and Klobuchar and Representatives Matsui and Wenstrup:

As the leading national organization that advocates for individuals with spinal muscular atrophy (SMA) and their families, **Cure SMA is pleased to support the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act,** your bipartisan legislation to increase the patient voice in the development and approval of life-saving treatments and cures.

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 benefited from past Congressional efforts to elevate patient voice, perspective, and experience within the drug development and approval process. Cure SMA and the SMA community have met multiple times since 2017 with the U.S. Food and Drug Administration (FDA), including a Patient-Focused Drug Development (PFDD) meeting where individuals with SMA and their caregivers described their experiences living with the disease and their goals and needs for future research and treatments.

Past patient engagement has helped to inform FDA officials about the SMA community's expectations and priorities for current and future treatments for SMA. For example, at one of the FDA engagement meetings, an adult with SMA described how his current SMA treatment has given him the ability to raise his elbows an additional couple inches off his wheelchair armrest, allowing him to eat independently rather than requiring the assistance of a caregiver. Without that additional patient-specific context, this life-altering improvement may appear inconsequential to someone without SMA.

Your BENEFIT Act legislation (S. 373 / H.R. 4472) continues to prioritize the patient community and their perspective by requiring the FDA to consider relevant PFDD information, including

patient-reported data, in the risk-benefit assessment framework used in the drug approval process. Current law does not require the FDA to consider patient perspective when assessing expected benefits versus potential risks of a potential treatment. This change will ensure that individuals with SMA and other patients are heard during this critical step in the drug approval process and that regulators understand disease impact on patients and what patients prioritize in the treatment of their disease.

Thank you for your leadership and efforts to ensure that research and approval of drug breakthroughs meet the needs of individuals with SMA and others. We support your efforts to advance this important provision 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,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President of Policy & Advocacy
Cure SMA