Make today a breakthrough.

February 17, 2023

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

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

Dear Senators Wicker and Klobuchar and Representatives Wenstrup and Matsui:

As the national organization that represents individuals with spinal muscular atrophy (SMA) and their families, **Cure SMA is pleased to support the reintroduction of the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act.** Your legislation will help to ensure that the U.S. Food and Drug Administration (FDA) continues to fully consider patient perspective in the development and approval of life-saving treatments, cures, and medical devices.

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 appreciates your efforts to elevate patient voice, perspective, and experience within the drug development and approval process. In many ways, the FDA's engagement with the SMA community can be considered a best practice that should be replicated and mandated. The FDA has hosted Cure SMA and members of the SMA community multiple times since 2017 to learn directly from individuals with SMA and their caregivers about their everyday living challenges and goals for future research and treatments. Most recently, Cure SMA participated in a Patient-Led Listening Session with FDA representatives from the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) in August 2022. During this virtual session, FDA officials heard directly from adults with SMA, a teenager with SMA, and parents of children with SMA about the need for new research and therapies to treat SMA. *"For me a treatment that targets muscle gain would greatly improve my independence and would ensure that I can continue doing the things that I love like power soccer,"* said a 16-year-old girl with SMA. An adult with SMA described how small improvements in everyday activities, such as putting her hair in a ponytail or lifting a half-gallon carton of milk out of the refrigerator to cook, would help increase her independence and well-

being. *"These things don't take a ton more muscle, but they are all muscle I still don't have. If science can create a safe effective treatment that helps me maintain, I fully believe and expect it will be able to create a combination treatment to allow me to gain some strength."* It is critically important that the FDA learn directly from the patient community about unmet needs and consider real world evidence and other patient-reported data when evaluating current products and treatments as well as those still in development.

Your BENEFIT Act legislation would prioritize the patient community and their perspective by requiring the FDA to consider relevant patient-focused drug development (PFDD) information in the risk-benefit assessment framework used in the drug approval process. Current law does not require the FDA to consider patient perspective, including patient-reported data, 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 the 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 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,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President of Policy & Advocacy
Cure SMA