Make today a breakthrough.

March 27, 2025

The Honorable Michael T. McCaul
U.S. House of Representatives
2300 Rayburn House Office Building
Washington, DC 20515

The Honorable Markwayne Mullin
U.S. Senate
330 Hart Senate Office Building
Washington, DC 20510

The Honorable Debbie Dingell
U.S. House of Representatives
102 Cannon House Office Building
Washington, DC 20515

The Honorable Michael Bennet
U.S. Senate
261 Russell Senate Office Building
Washington, DC 20510

Dear Representatives McCaul and Dingell and Senators Mullin and Bennet,

On behalf of individuals and families impacted by spinal muscular atrophy (SMA), **Cure SMA applauds your efforts to extend the Rare Pediatric Disease Priority Review Voucher (PRV) program through the Give Kids a Chance Act** (H.R. 1262/ S. 932). The U.S. Food and Drug Administration's (FDA) PRV program, which expired at the end of 2024, has been a key tool in fostering research interest and treatment success in SMA, a rare neuromuscular disease that causes debilitating muscle and motor function loss.

When the PRV program was established in 2012, SMA was considered the leading genetic cause of infant death. Babies born with SMA Type 1, the most common form of the disease, often died before reaching their second birthday. Those who survived required aggressive multi-disciplinary care and high-cost interventions, including permanent ventilation, feeding tubes, and around-the-clock caregiving. While researchers had identified the cause of the disease (a faulty survival motor neuron gene), no treatments were approved or on the immediate horizon.

Thanks to PRV and related rare disease-focused policies and research investments, the prognosis for SMA babies born today with SMA has vastly improved. Individuals with SMA and their families have choices in available FDA-approved SMA treatments and hope for the future because of a robust research pipeline. Priority vouchers were provided for each of the current SMA treatments, which helps to incentivize further research and development in SMA and other rare diseases. Treatment access in the SMA community has resulted in decreased mortality rates, reduced hospitalizations, and increased function and quality of life. SMA is no longer considered the leading genetic cause of infant death, demonstrating the impact PRV and similar incentives have in driving innovation and saving lives. A **parent of a child with SMA** said, *"My six-year-old daughter was born with this disease and is only alive today because of a newly FDA approved treatment."*

The bipartisan Give Kids a Chance Act would renew the vital PRV policy through the end of fiscal year 2029 and provide Congress with a report analyzing the program's effectiveness. The extension of the PRV comes at a critical time for the SMA community. While current SMA treatments are effective at slowing or stopping future progression of the

disease, they do not cure the disease or restore lost muscles or motor function. It is critical that Congress prioritizes SMA incentives such as PRV and SMA-specific research investments through the Peer Reviewed Medical Research program at the U.S. Department of Defense to address the chronic health and independence challenges facing children and adults with SMA. *"The more research given to this disease, the more hope our children have to live longer, happier lives,"* said a **parent of a child with SMA**.

Cure SMA and the SMA community thank you for your work to extend this important drug development tool and for prioritizing research and innovation in SMA and other rare diseases. We look forward to working with you to help drive further breakthroughs that improve the health and well-being of individuals with rare diseases, including SMA. If you have any questions, your staff can reach out to Cure SMA through Maynard Friesz, Cure SMA Vice President for Policy and Advocacy, at 202-871-8004 or maynard.friesz@curesma.org.

Sincerely,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President, Policy
Cure SMA