Make today a breakthrough.

April 24, 2025

The Honorable Susan Collins
Chair
U.S. Senate Appropriations Committee
United States Senate
Washington DC 20510

The Honorable Patty Murray
Vice Chair
U.S. Senate Appropriations Committee
United States Senate
Washington DC 20510

Dear Senators Collins and Murray,

On behalf of individuals and families impacted by spinal muscular atrophy (SMA), **Cure SMA thanks you for holding a hearing on biomedical research and the breakthroughs it produces**. Cure SMA is pleased to share the SMA community's perspective on how federal biomedical research at the U.S. Department of Health and Human Services (HHS) and U.S. Department of Defense (DOD) have saved and improved lives of children and adults with SMA and why it is critical that federal investments in SMA and other biomedical research are prioritized in fiscal year (FY) 2026.

SMA is a rare neurodegenerative disease that causes severe muscle weakness and motor function loss. The disease impacts residents and families in all 50 states. In addition, about one in 50 Americans is a SMA carrier.[i] When both parents are SMA carriers, every child they have together has a 25 percent chance of having SMA.

Cure SMA was founded forty years ago by parents of children with SMA to find a cure for SMA and to support other families impacted by the disease. At the time, SMA was the leading genetic cause of infant death. Babies born with SMA Type 1 often died before reaching their second birthday due to respiratory failure. *"Our son appeared to be a perfectly healthy baby. However, warning signs started to appear shortly after we took him home,"* said an **Arkansas mother whose son with SMA** died in 2011 when he was only 87 days old. Children with SMA that survived often required around-the-clock caregiving and aggressive medical care, including high-cost hospitalizations, ongoing therapies, and specialized equipment and services.

Past private and public research investments, including through the HHS's National Institutes of Health[ii] and DOD's Peer-Reviewed Medical Research Program,[iii] have led to three U.S. Food and Drug Administration-approved SMA treatments,[iv] a robust treatment pipeline,[v] and discoveries across numerous other neurological and rare diseases.[vi] Existing SMA treatments slow or stop future nerve damage associated with SMA. If delivered early, before the onset of symptoms, babies born today with SMA are thriving and achieving key developmental milestones such as walking.[vii] Because of biomedical research breakthroughs, the SMA mortality rate has decreased by nearly 80 percent since 2014.[viii] SMA is no longer the number one genetic cause of infant death. *"SMA is a terrible diagnosis that would kill kids by the time they are two. Instead, our kids are alive and thriving thanks to therapies that were developed through federal research,"* said a **Georgia mother of two children with SMA**, ages 2 and 4.

While significant SMA health needs remain, SMA is widely considered a biomedical research success story, thanks to our country's public-private biomedical research model. Private funding, including more than $17.5 million by Cure SMA and the SMA community since 2004, often provides the earliest seed money for promising research proposals. Cure SMA has awarded more than 140 basic research grants in the last two decades to explore novel research projects. Scientists and researchers can leverage the learning from early private funding to secure federal research grants for the next phases of research and development.

Each of the SMA treatments followed this successful private-public model. For example, the first SMA treatment—Spinraza (nusinersen)—was launched with early private research, including from Cure SMA (2003 to 2006).[ix] Federal research investments helped identify a treatment strategy and target and facilitated later stage translational and clinical research.[x] This private-public biomedical research model has greatly changed the trajectory of SMA. *"I am living proof that NIH-funded research saves lives,"* a **young adult with SMA** who serves on Cure SMA's Adult Advisory Council wrote about the impact federally funded university research has had on him and others in the SMA community.

In addition to benefiting individuals with SMA, SMA-focused research has also led to breakthroughs in other areas. The antisense oligonucleotide (ASO) delivery mechanism used for the first SMA treatment spurred research discoveries for Huntington's disease[xi] and other neurological diseases[xii] and the 2019 approval of gene therapy for SMA led to gene therapy treatments for other rare diseases, including Duchenne muscular dystrophy.[xiii] Cure SMA and the SMA community are grateful for past federal research investments that have contributed to these successes. We agree with Chair Collins' statement that *"there is no investment that pays greater dividends to American families than our investment in biomedical research."*[xiv]

**Cure SMA and the SMA community respectfully urge Congress to protect the private-public biomedical research model and federal investments and prioritize new research in SMA**. While current SMA treatments help slow or stop future degeneration, they do not cure the disease or reverse neurological damage or muscle loss that occurred before taking a treatment. Children and adults with SMA who were born before SMA treatments face chronic health challenges and barriers to independence due to muscle and motor function loss that occurred before accessing an SMA treatment. These individuals, which represent the largest segment of the SMA population, suffer from severe fatigue, muscle weakness, motor function loss, and other pervasive challenges that they hope future research and treatments will address. A **Maine mother of an 8-year-old second-grader with SMA** said, **"***New SMA research is needed to address the chronic health and independence challenges facing my son and other children and adults with SMA."* A **Washington State mother of a 22-year-old with SMA** said, *"Our daughter is currently living at home while working to improve the status of her many health issues. She would love nothing more than to be able to live an independent life."*

Early SMA research and development focused on SMA genetics. The next wave of SMA research will focus on nerves, muscles, neuromuscular junctions, and related targets that also have broad applicability beyond SMA. For example, researchers are now examining the use of spinal cord stimulation to improve muscle function in individuals with SMA given SMA affects the

motor nerve cells in the spinal cord.[xv] This early research, which was recently featured on Fox News[xvi] and NPR,[xvii] could benefit military warfighters and veterans who experienced nerve damage, spinal cord injuries, and related muscles disorders during their service to our country. To accelerate promising research focused on regenerating nerves, strengthening muscles, and restoring motor function, **Cure SMA's top fiscal year 2026 priority is to reinstate "spinal muscular atrophy" as a priority research area in DOD's Peer-Reviewed Medical Research Program.**[xviii] Congress last included SMA as a priority area through report language in the FY 2020 Conference Report.[xix]

Cure SMA and the SMA community are concerned about the impact federal research funding changes[xx] and cuts[xxi] could have on meeting the critical research needs of the SMA community. The Full-Year Continuing Appropriations and Extensions Act of 2025, for example, included a significant cut to the DOD medical research program that resulted in the Peer-Reviewed Medical Review Program being cut from $370 million in FY 2024[xxii] to only $150 million in FY 2025.[xxiii] **In addition to seeking report language adding SMA to DOD Peer-Reviewed Medical Research Program, Cure SMA and the SMA community supports the full restoration and protection of biomedical research across HHS and DOD to ensure future breakthroughs.**

Cure SMA and the SMA community that we represent appreciates your past support of federal biomedical research that has significantly improved the lives of children and adults with SMA and their families. Fostering innovation today through biomedical research will help lead to tomorrow's breakthroughs in SMA and other conditions. Thank you for considering the SMA community's experience related to biomedical research. If you or your staff have questions or need additional information, please contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202-871-8004 or maynard.friesz@curesma.org.

Sincerely,

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Cc: Members of the U.S. Senate Appropriations Committee

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