Written Testimony for the Record

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in support of

Spinal Muscular Atrophy Research

Chairman Aderholt, Ranking Member DeLauro, and Members of the Subcommittee,

Thank you for the opportunity to provide written testimony on behalf of individuals with spinal muscular atrophy (SMA) in support of SMA research at the National Institutes of Health (NIH). Cure SMA, which represents individuals and families with SMA in Alabama, Connecticut, and across the country, respectfully requests your support in rejecting proposed cuts to NIH funding and including report language that urges continued focus on SMA research at the National Institute of Neurological Disorders and Stroke (NINDS).

SMA is a progressive neurodegenerative disease that robs individuals of physical strength by damaging motor nerve cells in the spinal cord, impairing their ability to walk, swallow, and—in the most severe cases—breathe. SMA affects approximately 1 in 15,000 births in the United States, and 1 in 50 Americans is a carrier. When both parents are SMA carriers, each child they have together has a 25% chance of having SMA. The disease impacts children and adults in all 50 states.[i] As one **father of two children living with SMA** shared, *"Every day we wonder what their lives will look like, just like every parent does. Except our wonders are heavily affected by the fears that their bodies might not support them through the whole journey."*

Historically, individuals with SMA required intensive, multidisciplinary care to survive. The most severely affected often depended on permanent ventilation, feeding tubes, and around-the-clock medical support. Before treatments were available, babies born with SMA Type 1, the most common and severe form, often did not live past their second birthday.[ii] A **parent of a child born with SMA Type 1 in 2010** said*, "Our doctor gave us the unfortunate news that our son was going to die. Two months later, he passed away. He was five months and three days old. It was agonizing for our family."*[iii]

Today, the outlook for SMA has changed dramatically, thanks to groundbreaking treatments made possible by years of public and private research[iv] SMA is no longer considered the leading genetic cause of infant death. Babies born with the most severe form are now living and reaching developmental milestones that were once unimaginable. In the past decade alone, the SMA mortality rate has decreased by 60%, and reliance on intensive medical equipment and specialized care has declined significantly, especially for infants treated before symptoms appear. A **grandmother of a child with SMA** shared, *"Thanks to SMA research, my granddaughter received a cutting-edge genetic treatment and is presently a healthy, very active six-year-old. I can't even begin to express my gratitude!"*

Despite this progress, significant unmet needs remain. Many individuals with SMA experienced irreversible nerve damage and loss of muscles and motor function before treatments became available. Current therapies do not restore that lost function. As a result, individuals who have lost the ability to walk, eat independently, or perform daily tasks have no treatment options to regain those abilities.

Notably, approximately 75% of the U.S. SMA population is age 13 or older—individuals who experienced disease progression before access to treatment.[v] A **28-year-old woman with SMA said**, *"Losing even a small amount of strength can mean losing the ability to perform a task independently. For example, I recently lost the ability to take communion at church without assistance—an experience that meant a great deal to me. Moments like these are deeply emotional reminders of the progression of the disease."*

Additional research is critical to address these gaps. Advances targeting these secondary effects of SMA are needed to regenerate nerve cells, rebuild muscle, and restore lost function. A **34-year-old adult with SMA** who works as an office assistant said, *"With improved strength, I could do more physically. I could type faster or type for longer periods without getting tired. Or whether at home or out at a restaurant, I could lift the heavy cups or heavy silverware without struggling."* A **parent of two middle school-aged children with SMA** emphasized the need for new treatments *"to minimize the neuromuscular decline and complications of this disease and promote better physical outcomes and quality of life."* She added, *"We look ahead to additional research for treatments not only for our children, but for those with further-advanced SMA who were older when the first treatments became available."*

Cure SMA and the broader SMA community appreciate this Subcommittee's long-standing bipartisan commitment to biomedical research. We are grateful that the Consolidated Appropriations Act of 2026 provided $48.7 billion for NIH,[vi] as well as robust funding for NINDS, which played a key role in identifying the genetic cause of SMA and advancing early research that led to today's treatments.[vii]

However, continued progress depends on sustained investment and prioritization. Cure SMA respectfully urges the Subcommittee to reject the proposed NIH funding cuts in the President's FY 2027 Budget[viii] and to retain the following report language encouraging NINDS to maintain its commitment to addressing the needs of individuals with SMA. Given recent leadership and staffing changes at NINDS, it is especially important that this Subcommittee preserve this language in the FY 2027 Labor, Health and Human Services, and Education Appropriations Report.

> **Spinal Muscular Atrophy.—**The Committee encourages continued NIH research into spinal muscular atrophy (SMA), a neuromuscular disease that causes degenerative nerve damage and results in severe muscle loss and impaired motor function. The Committee is aware that past NINDS research has led to greater understanding of the nervous system and contributed toward SMA treatments that slow or stop future nerve damage. The Committee also recognizes that current treatments do not cure the disease or reverse its debilitating symptoms. Without additional SMA research, the Committee is concerned that adults and children with SMA who were born after treatments and early diagnosis were available will continue to face chronic health challenges and significant barriers to independence. Furthermore, it is not yet fully known the extent of need among children treated prior to symptom onset. As such, the Committee encourages NINDS to expand its research in SMA into the role and function of survival motor neuron (SMN) protein, investigation of non-SMN pathways and targets capable of modifying disease, and how to combine SMN-enhancing and non-SMN approaches for optimal therapeutic outcomes.

I will close with the words of **one SMA community member**: *"The hope for the future has already been kindled by treatments made possible through past research. I am hopeful that continued research will drive even more discoveries—not only for individuals with SMA, but for others living with conditions that affect mobility and independence."*

Thank you for your support of biomedical research and for considering the views and requests of Cure SMA and the SMA community. We would be happy to answer any questions or provide additional information as you consider this request.

[i] SMA Signs and Symptoms, John Hopkins Medicie, 2026: https://www.hopkinsmedicine.org/health/conditions-and-diseases/spinal-muscular-atrophy-sma
[ii] SMA Signs and Symptoms, John Hopkins Medicie, 2026: https://www.hopkinsmedicine.org/health/conditions-and-diseases/spinal-muscular-atrophy-sma
[iii] SMA Parent Testimony, Nebraska Legislative Committee Hearing, 2020: https://nebraskalegislature.gov/FloorDocs/106/PDF/Transcripts/Health/2020-01-22.pdf
[iv] Nusinersen (Spinraza) – SMA, National Institute of Neurological Disorders and Stroke, 2025: https://www.ninds.nih.gov/about-ninds/what-we-do/impact/ninds-contributions-approved-therapies/nusinersen-spinrazar-spinal-muscular-atrophy-sma
[v] State of SMA, Cure SMA, https://www.curesma.org/publications/#stateofsma
[vi] Consolidated Appropriations Act of 2026: https://www.congress.gov/119/crec/2026/01/22/172/15/CREC-2026-01-22-bk2.pdf
[vii] SMA, NINDS, 2026: https://www.ninds.nih.gov/health-information/disorders/spinal-muscular-atrophy
[viii] FY 2027 Budget of the U.S. Government, OMB, 2026: https://www.whitehouse.gov/wp-content/uploads/2026/04/budget_fy2027.pdf