March 18, 2025

The Honorable Robert Aderholt
Chairman
Labor-HHS-Education Subcommittee
United States House of Representatives
Washington, DC 20515

The Honorable Rosa DeLauro
Ranking Member
Labor-HHS-Education Subcommittee
United States House of Representatives
Washington, DC 20515

Dear Chairman Aderholt and Ranking Member DeLauro,

Cure SMA, which represents individuals and families affected by spinal muscular atrophy (SMA), appreciates your leadership and continued support for biomedical research, including your recent remarks at the National Institutes of Health (NIH) hearing. SMA, a debilitating neuromuscular disease, reflects both the life-changing impact of NIH-funded research and the urgent need for continued investment to address the significant unmet needs that remain in the SMA community.

During your subcommittee hearing, NIH Director Dr. Jay Bhattacharya highlighted gene therapy advancements developed through NIH-funded research, including one for a previously rare, fatal disease that he noted has given "baby KJ" the promise of a long and healthy life.[i] Children with SMA have similarly benefited from NIH-supported discoveries. SMA is a rare neuromuscular disease that causes irreversible nerve damage and severe muscle loss.

Less than a decade ago, SMA was the leading genetic cause of infant death. Babies born with the most common and severe form of the disease often did not survive past their second birthday due to respiratory failure and other complications. Today, thanks to research funded by both Cure SMA and the NIH, multiple treatments, including gene therapy, are helping to slow or even halt disease progression.

When these treatments are delivered early, before symptoms appear, as is increasingly possible through nationwide newborn screening, outcomes can be remarkable. Babies with the most severe genetic forms of SMA are now reaching developmental milestones and thriving. One such example is Noah, who was diagnosed with SMA within 48 hours of birth through newborn screening and received gene therapy within his first three weeks of life, before symptoms began.

Today, Noah is four years old and free of SMA symptoms. He swims, rides his scooter, runs, jumps on his trampoline, and goes sledding in the winter, even thinking about skiing. He loves playing with cars, wrestling with his brother Theo, and dancing to Danny Go videos. **Noah's strong outcome is a direct result of biomedical breakthroughs supported by your Labor, Health and Human Services, and Education Appropriations Subcommittee**. The National Institute of Neurological Disorders and Stroke (NINDS) funded the foundational research that identified the genetic cause and mechanisms of SMA,[ii] while Cure SMA supported early gene therapy research that ultimately led to the FDA-approved treatment Noah received.[iii]

While Noah's story is increasingly common among children diagnosed at birth today, most individuals living with SMA in the United States were born before these treatment breakthroughs. Many of these children and adults experienced irreversible nerve damage, muscle loss, and other long-term complications before accessing treatment. Current therapies are most effective when delivered early and cannot reverse damage that has already occurred. As a result, many people with SMA continue to live with significant physical limitations—approximately 73 percent use a

power wheelchair, and 85 percent of adults rely on a caregiver for daily support.[iv] **The SMA community is urgently seeking future treatments that can reverse muscle loss, restore nerve damage, and regain motor function—goals also shared by others with neuromuscular and related disorders.**

Cure SMA is committed to advancing this work by investing in early-stage research. Earlier this year, we announced $750,000 in new SMA research grants, including a project at Yale University focused on non-viral delivery of base-editing therapy. [v] These seed investments often enable researchers to secure additional NIH funding, strengthening the public-private partnership that has already led to today's treatments and will drive tomorrow's breakthroughs.

As such, Cure SMA and the broader SMA community respectfully request your continued support for report language in the fiscal year 2027 Labor-HHS-Education Appropriations bill to highlight these unmet needs and prioritize SMA research at NINDS and across NIH. This language is especially important given recent staffing changes at NIH, including at the institute leadership level. **Cure SMA respectfully requests your support for the following language in FY 2027:**

> 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.

We also support your efforts to continue increasing funding for NIH, particularly for research that leads to meaningful outcomes for individuals living with rare diseases. We commend your commitment to fostering the next generation of scientists by providing greater funding stability, expanding grant opportunities, and encouraging innovative, high-impact research.

Thank you for considering the views of Cure SMA. Please do not hesitate to reach out if you or your staff have any questions or would like additional information. They can contact Cure SMA at through maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

Kenneth Hobby
President

Jacqueline Glascock, PhD
Vice President, Research

Maynard Friesz
Vice President, Policy

[i] Thanks to @NIH-supported research, baby KJ successfully received personalized gene therapy treatment for CPS1: https://x.com/NIHDirector_Jay/status/1923537543586296035
[ii] NINDS Funded Research: https://www.ninds.nih.gov/sites/default/files/documents/sma_design_508c.pdf
[iii]Cure SMA funds Nationwide Children's Hospital Gene Therapy Grants: https://www.curesma.org/zolgensma/#history
[iv] Cure SMA State of SMA Report: https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf
[v] Cure SMA Awards SMA Research Grants, 2026, https://www.curesma.org/cure-sma-awards-75000-grant-to-jiangbing-zhou-phd-at-yale-university/