cure SMA. Make today a breakthrough.

June 16, 2026

Office of Federal Financial Management
Office of Management and Budget
Executive Office of the President
Washington, DC 20503

Re: Comments on Proposed Rule — Regulation for Federal Financial Assistance (2 CFR Parts 1, 25, 170, et al.), RIN 3112-AA07

Dear Office of Federal Financial Management:

As the national organization dedicated to the treatment and cure of spinal muscular atrophy (SMA), Cure SMA is pleased to share the perspective of the SMA community, including individuals and families affected by SMA as well as the research community, on the proposed rule that would change how the National Institutes of Health (NIH) and other federal agencies review and award federal grants.[i] The lives of children and adults with SMA have greatly improved because of treatment and research breakthroughs developed through the current grantmaking process. We advise caution on changes that could inadvertently slow, limit, or halt scientific progress in developing treatments and a cure for SMA.

SMA is a debilitating neuromuscular disease that causes irreversible nerve damage and significantly weakens the muscles that control movement, breathing, and everyday life. SMA affects children and adults in all 50 states. Historically, babies born with SMA Type 1, the most common and severe form of the disease, died before reaching their second birthday.

Thanks to research breakthroughs developed through federally awarded grants,[ii] multiple FDA-approved treatment options now exist for children and adults with SMA that slow or halt future muscle deterioration associated with the disease. SMA is no longer considered a leading genetic cause of infant death. The SMA mortality rate has dropped nearly 60 percent in the last decade.[iii] Children born with the most severe form of the disease who access treatment within days of birth are thriving, with 100 percent walking by age two when treated within two weeks of birth.[iv] In addition, hospitalization rates are declining and quality of life is improving for children and adults with SMA.[v] As **one adult with SMA** shared, *“I have benefited greatly from SMA research and treatments that have slowed the progression of this deadly disease, resulting in less intensive and fewer health-related medical visits.”*

These improvements would not have been possible without the research advancements developed through federal grant awards. **The federal government’s long-standing process of annual award competitions decided by scientific peer review panels has proven successful in SMA, as demonstrated by the extraordinary progress of the last decade.**

Despite these biomedical breakthroughs, significant unmet need remains, particularly among older children, teens, and adults with SMA who did not benefit from early access to treatment. About 78 percent of individuals reported needing a wheelchair or other durable medical equipment[vi] and 87 percent of individuals with SMA require one or more caregivers to assist with routine daily activities.[vii] This group, which represents the largest segment of the U.S. SMA population,[viii] is seeking new federal research into treatments that could help them gain muscle strength, achieve new motor function, reduce fatigue, improve fine motor skills, improve respiratory function, decrease dependency on wheelchairs and other equipment, improve swallowing, and improve voice strength, among other unmet needs. Past research innovation gives the SMA community enormous hope for the future. An **adult with SMA** who lost her ability to walk in her late 30s said, *"A few years ago I began treatment for my SMA. It has been successful, but I still haven't regained the ability to walk. Researchers are close to making this happen."* **A parent of a 10-year-old boy with SMA** said, *"we have so much more that is needed. Additional therapies and hopefully one day a cure would mean so much to my family and my son's future!"*

The SMA community's hope for the future depends on a consistent, rigorous, and reliable federal research system. The federal government remains the largest funder of biomedical research in the world.[ix] SMA researchers who develop scientific ideas through Cure SMA and other private funds compete for federal grants to advance the promising research through the development pipeline. These researchers, and the SMA community they support, rely on the federal government's scientific and performance-based annual process to ensure that their ideas are fairly considered based on the scientific merit. The current system works, as evidenced by the progress of the last decade.

Cure SMA cautions against changes to the current federal grant review and award system that could compromise future success in SMA research. While we support the goals of transparency, accountability, and efficiency in federal grantmaking, we are concerned that certain provisions of the proposed rule, as currently drafted, would undermine the features of the federal biomedical research grant process that have made it so effective – not only for SMA, but for countless other diseases that depend on the same system of annual, peer-reviewed, merit-based federal investment.

Thank you for considering the views of Cure SMA and the individuals and families we represent. We hope that by maintaining the scientific integrity of the federal grant process, we can together continue to improve the health and independence of children and adults with SMA. Please do not hesitate to reach out to Cure SMA should you have further questions about SMA or how the current federal awards system has benefited the people we serve. Cure SMA can be reached at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

K.A. Hobby
Kenneth Hobby
President

Jacqueline Glascock
Jacqueline Glascock, PhD
Chief Scientific Officer

Maynard Friesz
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[i] Federal Register, 2026: https://www.federalregister.gov/documents/2026/05/29/2026-10817/regulation-for-federal-financial-assistance

[ii] National Institute of Neurological Disorders and Stroke, 2026: https://www.ninds.nih.gov/about-ninds/what-we-do/impact/ninds-contributions-approved-therapies/nusinersen-spinrazar-spinal-muscular-atrophy-sma

[iii] Cure SMA State of SMA Report, 2025: https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf#page=50

[iv] Cure SMA State of SMA Report, 2023: https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf#page=31

[v] Cure SMA State of SMA Report, 2025: https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf#page=43

[vi] Cure SMA State of SMA Report, 2025: https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf#page=31

[vii] Cure SMA State of SMA Report, 2025: https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf#page=35

[viii] Cure SMA State of SMA Report, 2025: https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf#page=10

[ix] National Institutes of Health, 2026: https://www.nih.gov/grants-funding