



ADDRESS THE UNMET NEEDS OF CHILDREN AND ADULTS WITH SPINAL MUSCULAR ATROPHY

Support National Institutes of Health Funding and Research into SMA

DID YOU KNOW?

- Spinal muscular atrophy (SMA) is an inherited neurodegenerative disease that affects the motor nerve cells in the spinal cord and impacts the muscles used for activities such as breathing, eating, crawling, and walking. SMA impacts 1 in 11,000 births in the U.S. ([Source: Cure SMA](#))
- The National Institutes of Health (NIH), the premiere medical research agency in the world, collaborates with multiple partners to speed up the development of treatments for rare diseases, such as SMA. Past NIH research has led to effective disease-modifying treatments that target the underlying cause of SMA and slow or stop further degeneration. ([Source: National Institute of Neurological Disorders and Stroke](#))
- Individuals with SMA and their families continue to report significant challenges, including muscle weakness, fatigue, severe scoliosis, and joint contractures that they hope future treatments will address. ([Source: Voice of the Patient Report \(Cure SMA\)](#))
- Cure SMA, the national organization focused on finding a cure for SMA, has self-funded more than \$35 million in SMA research in the last two decades. However, fewer than 25 percent of promising research proposals were funded in 2023 due to limited private funds. ([Source: Cure SMA](#))

CURE SMA POSITION

To help address the ongoing and significant unmet needs of individuals with SMA, Cure SMA urges Congress to fully fund the NIH and include report language in the Labor, Health and Human Services, Education, and Related Agencies Appropriations bill to direct NIH to support new research into the role and function of survival motor neuron (SMN) protein, investigation into non-SMN pathways and targets capable of modifying disease, and research into how to best combine SMN-enhancing and non-SMN approaches for optimal therapeutic outcomes.

WHY CONGRESS SHOULD ACT

Past congressional investments and policies have helped to spur discoveries into SMA. Current SMA treatments can slow or stop future degeneration associated with SMA. If delivered early, especially before the onset of symptoms, these treatments can greatly improve motor and developmental gains and reduce future need for intensive health care and specialized supports. Past public and private research in SMA has also yielded new understanding of the nervous system and disease mechanisms that benefit other neurological and neuro-muscular diseases. However, current SMA treatments do not cure the disease or its debilitating symptoms. Significant unmet needs remain across all ages and disease stages of SMA. Privately funded SMA research is not keeping up with research need or viable proposals. Continued NIH research into SMA is needed to meet research demand and address ongoing challenges, including muscle weakness and fatigue, that affect people with SMA and other nervous system disorders.



Cure SMA Advocacy | 800-886-1762 | advocacy@cureSMA.org | cureSMA.org

Cure SMA is a national organization that advocates for individuals with spinal muscular atrophy, a progressive neurodegenerative disease that robs people of physical strength, taking away their ability to walk, swallow, and breathe.

LABOR-HHS-EDUCATION APPROPRIATIONS REQUEST INFORMATION

Appropriations Bill:	Labor-HHS-Education
Federal Department:	U.S. Department of Health and Human Services
Agency/Account:	National Institutes of Health (Office of the Director)
Cure SMA Request:	Report Language (see below)

CURE SMA REPORT LANGUAGE REQUEST:

Spinal Muscular Atrophy.—The Committee remains committed to 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. Past SMA research at NIH, particularly through NINDS, has led to disease-modifying SMA treatments and greater knowledge of the nervous system, which has benefited other neurological and neuro-muscular disorders. While current SMA treatments slow or stop future degeneration, they do not cure SMA. Individuals with SMA, particularly adults, the largest segment of the SMA population, face significant challenges in muscle weakness and fatigue due to degeneration that occurred prior to treatment. Individuals treated prior to clinical symptoms onset may also display unmet needs, such as bulbar impairment and gait abnormalities. The Committee urges NIH to address the significant unmet need that exists across all ages and disease stages of SMA by supporting new SMA research into the role and function of survival motor neuron (SMN) protein, investigation into non-SMN pathways and targets capable of modifying disease, and research into how to best combine SMN-enhancing and non-SMN approaches for optimal therapeutic outcomes.



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