



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](#))
- Children and adults with SMA and their families seek new treatments to address persistent and significant unmet needs, including muscle strength (97% of adults), motor function (91%), fatigue (83%), and respiratory function (63%). ([Cure SMA Community Survey](#))
- 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 expand its research in SMA to address fatigue, muscle weakness, motor function loss, and other unmet needs that are pervasive among adults and older children with SMA, the largest segment of the SMA community.

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 (National Institute of Neurological Disorders and Stroke)
Cure SMA Request:	Report Language (see below)

CURE SMA REPORT LANGUAGE REQUEST:

Spinal Muscular Atrophy.—The Committee commends NIH for its continued support of research in spinal muscular atrophy (SMA), a rare neuromuscular disease. The Committee is aware that past NINDS research has led to greater understanding of the nervous system and contributed toward approved 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, we do not fully know the extent of need among children treated prior to symptom onset. As such, the Committee encourages NINDS to expand its research in SMA to address fatigue, muscle weakness, motor function loss, and other unmet needs that are pervasive among the largest segment of the SMA community. Additional research into these common needs may also benefit individuals with other neuromuscular and rare diseases who face similar challenges.



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