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 effective treatments into rare diseases, such as SMA. (Source: NIH)
- Past NIH research has contributed toward the development of effective disease-modifying treatments that target the underlying cause of SMA and slow or stop further degeneration. Additional SMA research is needed for individuals with SMA to restore strength and reverse SMA symptoms. (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 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 SMA research 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. Continued research into SMA will help to address ongoing challenges, including muscle weakness and fatigue, that affect people with SMA and other nervous system disorders.

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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 commends NIH for its past research into spinal muscular atrophy (SMA) that has led to new therapies to treat SMA and also contributed toward greater knowledge and research capacity into nervous system disorders. While current SMA treatments can slow or stop future degenerative nerve damage, they are not cures and there remains significant unmet need across all ages and disease stages of 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 these unmet needs, which are common across other neurological and neuro-muscular diseases, by supporting 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.