



ENSURE FULL AND TIMELY ACCESS TO LIFESAVING TREATMENTS

Support the Bipartisan Safe Step Act of 2021 (H.R. 2163 / S. 464)

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 U.S. Food and Drug Administration (FDA) has approved three treatments for SMA—Spinraza (2016), Zolgensma (2019), and Evrysdi (2020)—that make it possible for individuals with SMA today to stop the progression of the disease, improve motor function, achieve developmental milestones, and live independently and contribute to their communities. ([Source: Cure SMA](#))
- Research shows that SMA treatments are most effective when delivered early, and especially when treated before symptom appear, given the rapid degeneration of the disease that affects motor neurons needed for muscle strength. Lost motor neurons cannot be restored. ([Source: National Center for Biotechnology Information](#))
- Most families of children with SMA (60%) and adults with SMA (59%) have reported that they have had to appeal an insurance denial related to SMA treatment coverage. ([Source: Cure SMA Community Survey](#))

WHY CONGRESS SHOULD ACT

Health plans that include step therapy protocols require patients to try the plan's preferred treatment before the patient can access the desired treatment originally prescribed by their physician. Step therapy protocols are aimed at controlling spending on medications and may ignore a patient's unique circumstances, medical history, and choice. For degenerative diseases such as SMA, the use of restrictive protocols and other barriers that delay access to the most clinically appropriate treatment can be harmful to the patient's overall health and well-being. For example, infants with SMA

Type 1, the most common and severe form of the disease, lose 90% of their motor neurons before reaching 6 months of age. Lost neurons can never be regenerated, leading to neuromuscular and respiratory muscle weakness that can progress to life-long medical needs and costs, as well as be fatal.

Beginning the most clinically appropriate treatment as early as possible is the only way to prevent this motor neuron loss.

CURE SMA POSITION

To ensure individuals with SMA and others have full and immediate access to the treatment that is most appropriate to their clinical needs and goals, Cure SMA urges Congress to approve the **Safe Step Act of 2021 (H.R. 2163 / S. 464)**.

The legislation would:

- Require a group health plan to implement a clear and transparent process for a patient or physician to request an exception to a step therapy protocol.
- Create step therapy standard exemptions when:
 - The patient has already tried the medicine without success.
 - Delayed treatment will cause irreversible consequences.
 - The treatment is contraindicated or has caused or is likely to cause an adverse reaction.
 - The treatment has, will, or is likely to prevent a participant from fulfilling their occupational responsibilities at work or performing activities of daily living.
 - The patient is stable for his or her disease or condition on the current drug or treatment.
- Require a group health plan to respond to an exemption request within 72 hours in all circumstances, and 24 hours if the patient's life is at risk
- Incentivize states to pay family caregivers of children and adults with SMA and other disabilities.



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.