



## PROMOTE REPRESENTATION OF PEOPLE WITH RARE DISEASES

*Support the Creation of Rare Disease Advisory Councils in Every State*

### DID YOU KNOW?

- Rare diseases are defined as conditions that impact fewer than 200,000 people in the U.S. Collectively, there are approximately 7,000 rare diseases that affect 30 million Americans. Of the 7,000 rare diseases, only about 5 percent have FDA-approved treatments. ([Source: U.S. Government Accountability Office](#))
- 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. There are three treatments for SMA approved by the U.S. Food and Drug Administration. ([Source: Cure SMA](#))
- The economic burden of rare diseases in the U.S. is nearly \$1 trillion, which includes direct medical costs (57% of \$966 billion total) and indirect and nonmedical costs (43%). In addition, significant unmet need exists within the rare disease community. ([Source: EveryLife Foundation](#))
- The first Rare Disease Advisory Council was created in North Carolina in 2015 by patients, caregivers, families, and providers. ([Source: National Organization for Rare Disorders](#)).

### CURE SMA POSITION

Cure SMA urges all states to form a **Rare Disease Advisory Council (RDAC)** to give the rare disease community a stronger voice in the state and local issues that impact their lives.

The goal of a RDAC is to:

- Provide advice to the governor, legislature, and state agencies on issues that impact the rare disease community.
- Include broad membership that includes individuals with rare diseases and representatives of rare disease organizations.
- Host regular public meetings and forums to educate and advise leaders and the public on issues and proposals that impact the rare disease community.
- Develop public reports on rare disease proposals and topics, such as prevalence, unmet needs, strategies for diagnosis and care, and economic and social cost.

### WHY STATES SHOULD ACT

State policies and programs play a critical role in the lives of individuals with SMA and other rare diseases. The rare disease community should have a seat at the table when decisions are being considered and made that could impact them and their ability to live independently and access healthcare, educational supports, transportation, and other community services. In addition, state leaders and agency officials would benefit from a regular forum where stakeholders across government, healthcare, and the rare disease community could discuss opportunities and challenges and provide feedback on policy and program proposals.



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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.