



Statement of Support State Rare Disease Advisory Council (RDAC) Legislation

Rare Disease Advisory Council Legislation:

As the leading national organization that represents children and adults with spinal muscular atrophy (SMA), a rare genetic neuromuscular disease, Cure SMA fully supports legislation to establish Rare Disease Advisory Councils in every state. Because policies, programs, and actions made by state and local governments greatly impact individuals with SMA and other rare diseases, the rare disease community must have a seat at the table when those decisions are being considered and made. Rare Disease Advisory Councils fulfill the *Nothing About Us Without Us* promise by creating a regular public forum where key stakeholders across government, healthcare, and the rare disease community can discuss and make recommendations to governing bodies on issues that impact individuals with rare diseases and their ability to access essential care, treatment, and services that promote healthy and independent living.

Rare Disease Advisory Council Membership:

Cure SMA recommends that membership on Rare Disease Advisory Councils broadly represents the rare disease community by including young adult and adult self-advocates with rare diseases, parents or caregivers of children with rare diseases, and advocacy organization leaders that represent people with rare diseases. Rare Disease Advisory Council membership should also prioritize diversity to promote equitable access to quality health care for all, regardless of race, age, gender, or geography (i.e., urban/rural).

About Spinal Muscular Atrophy Background:

SMA is a rare progressive neurodegenerative disease caused by a mutation in the survival motor neuron gene 1. In a healthy person, this gene produces a protein that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, which can significantly impact an individual's ability to walk, swallow, and—in the most severe cases—even breathe. SMA impacts 1 in 11,000 births in the United States and an estimated 1 in 50 people is a genetic carrier. Three effective U.S. Food and Drug Administration-approved SMA treatments have changed the trajectory of the disease, making it possible for individuals with SMA to achieve unprecedented developmental milestones through early access to treatment. Newborn screening, which is now available to about 70% of all U.S. newborns, is the most effective way for babies with SMA to access timely treatments and available supports.

About Cure SMA:

Cure SMA is the leading national organization that represents individuals with SMA and their families across the United States. In addition to funding research into treatments and a cure for SMA, Cure SMA supports and advocates for children and adults with SMA by hosting educational conferences and workshops and managing equipment pools and other support programs. Cure SMA also educates healthcare providers and insurers about SMA, and advocates for policies and legislation that protect access to care, treatment, and services that promote independence and community living for all individuals with SMA.

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