



Make today a breakthrough.

February 28, 2022

Dear Senator:

On this **Rare Disease Day**, Cure SMA recognizes Congress for its efforts on behalf of children and adults with spinal muscular atrophy (SMA) and asks that you consider supporting Cure SMA's *Greater Independence* agenda priorities that will promote greater independence for all individuals with SMA.

SMA is a rare 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., and about 1 in every 50 Americans is a genetic carrier. If both parents are SMA carriers, every child they have together has a 25% chance of being diagnosed with SMA, regardless of race, ethnicity, and gender.

The SMA community has benefited from past congressional policy innovations and funding investments. All three U.S. Food and Drug Administration (FDA)-approved SMA treatments received fast-track consideration under the rare pediatric disease priority review authority established by Congress in 2012 and extended in 2020. In addition, children and adults with SMA may utilize services and supports through Medicaid and other federal programs to help them live, work, learn, and participate in their communities.

As the leading national organization that advocates for individuals with SMA and their families, Cure SMA asks that you stand with people with SMA on this day of awareness and change for the rare disease community by supporting the following legislative priorities:

- **Finalize the Fiscal Year 2022 Funding Bills:** The pending appropriations bills for the current fiscal year (FY) include key investments in newborn screening, IDEA education, disability employment, and accessible transportation, among other things, that promote independence and community living for individuals with SMA and other rare diseases. The inability to finalize FY 2022 funding would negate these strategic investments and revert to last year's funding levels.
- **Send the Newborn Screening Saves Lives Reauthorization Act to the President:** Early diagnosis and access to SMA treatment can lead to improved, long-lasting developmental outcomes for individuals with SMA. Newborn screening is key for babies with SMA to access timely treatments and available supports. Unfortunately, not all states screen for SMA, despite the 2018 federal recommendation. Final congressional passage of the Newborn Screening Saves Lives Reauthorization Act (which passed the U.S. House in June) will ensure states have the resources and technical assistance they need to screen for SMA and other heritable disorders.



- **Raise Patient Voice and Perspective in the Research, Development and Approval of New Treatments:** Increased muscle strength, new motor functions, and improved daily functioning remain top unmet needs of children and, especially, adults with SMA. New research and development are required to address these pressing SMA community needs. Establishing an Advanced Research Project Agency for Health (ARPA-H) and boosting patient-perspective and real-world evidence in the drug development and approval process, as proposed in Cures 2.0 and the BENEFIT Act, will help to ensure future treatments meet the SMA community's needs and expectations.
- **Help Individuals with SMA Access Medicaid Caregiving Services:** Many children and adults with SMA utilize in-home services to assist with daily activities, such as getting out of bed and dressed in the morning so they can get to work or attend school. However, many individuals with SMA struggle to find in-home caregivers or lack caregiving hours to meet their independent living needs. The temporary funding boost for Medicaid home and community-based services included in the American Rescue Plan must be increased and made permanent to ensure individuals with SMA and others can maintain their health and independence.

As an influential Member of Congress, you can help continue progress within the SMA community by supporting these Cure SMA *Greater Independence* agenda priorities. Your staff can reach out to Cure SMA at maynard.friesz@curesma.org or 202-871-8004 if they have questions related to these SMA community priorities.

Thank you for your consideration

Sincerely,

Kenneth Hobby
President
Cure SMA

Maynard Friesz
Vice President of Policy & Advocacy
Cure SMA