



Make today a breakthrough.

July 20, 2021

The Honorable Diana DeGette
United States House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Fred Upton
United States House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Upton:

As the leading national organization that supports and advocates for individuals with spinal muscular atrophy (SMA) and their families, **Cure SMA appreciates your leadership in developing a discussion draft for the 21st Century Cures 2.0 Act to help improve the health and research needs of the rare disease community.**

SMA is a genetic disease caused by a mutation in the survival motor neuron gene 1, or SMN1. 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 die, impacting an individual's ability to walk, eat, and breathe. 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. SMA impacts 1 in 11,000 births in the United States.

The SMA community has benefited from past congressional policies and investments aimed at spurring breakthrough treatments for rare diseases. The first-ever U.S. Food and Drug Administration (FDA) approved treatment for SMA received a rare pediatric disease priority review voucher through the FDA program established by Congress. The treatment was approved just 10 days after the 21st Century Cures Act was signed into law. Since, two additional SMA treatments have been approved, giving children and adults with SMA additional life-saving and life-improving options. While these treatments have revolutionized and dramatically changed the natural history of SMA, they do not represent a cure. There continues to be substantial unmet medical needs within the SMA community, especially for young adults and older adults with SMA. The SMA community recently identified new muscle strength, increased motor function, and improvements in activities of daily living as their top three unmet needs.

Given the role government investments and policies play in spurring research and development of treatment and cures, especially for rare diseases, **Cure SMA applauds your efforts and encourages Congress to swiftly consider these improvements and updates to the landmark 21st Century Cures Act.** Your Cures 2.0 discussion draft includes many important provisions and investments, such as the creation of the new Advanced Research Projects Agency for Health (ARPA-H), that will maintain our nation's focus on and leadership in accelerating biomedical breakthroughs, such as rare disease treatments and cures. Cure SMA is

pleased to highlight and comment on the following provisions (listed as they appear in the bill) that are especially impactful to the SMA community.

- **Sec. 103: Pandemic preparedness rare disease support:** Cures 2.0 would help address the challenges of individuals with rare diseases during public health emergencies through the development of pandemic preparedness plans. *Cure SMA supports the focus and attention on the needs of individuals with rare diseases during a public health emergency and asks that the plans include strategies for ensuring access to treatments and care, including in-home services.*
- **Sec. 201: Caregiver education and training:** Cures 2.0 would establish educational programs and training for family caregivers related to medication, nutrition, and other in-home services. Many individuals with SMA who utilize paid personal care attendants or in-home nurses may also receive assistance from family caregivers for their daily activities. Family caregiving duties increased during the COVID-19 pandemic when many individuals with SMA faced no or limited access to caregivers outside their home. *Cure SMA supports specialized education and training for family caregivers to compliment, not supplant the important role and need for full-time attendant care or in-home health services that some individuals with SMA may require.*
- **Secs. 204 & 304: Patient experience data and real world evidence:** Cures 2.0 recognizes the important roles of patient engagement, patient experience, and real world evidence in informing researchers, drug developers, and regulators throughout the drug development and approval process. *Cure SMA supports the use of patient experience data and real world evidence in the drug development and approval process, including the proposed establishment of a Real World Evidence Task Force of government and industry officials to promote patient engagement in the collection of this important data.*
- **Secs. 402 & 403: Increase telehealth coverage:** Cures 2.0 extends, integrates, and reviews the use of telehealth services within Medicare, Medicaid, and the Children's Health Insurance Program. Individuals with SMA and their families utilized telehealth services throughout the COVID-19 pandemic to access healthcare. Telehealth services can improve access to healthcare and specialists, especially for individuals with SMA living in rural or remote areas. However, some SMA families also reported missed diagnoses due to the use of virtual, rather than in-person, wellness checkups. *Cure SMA supports greater flexibility and expanded use of telehealth services, when appropriate and in the best interest of the patient.*
- **Sec. 404: Coverage and Payment of Breakthrough Devices:** Cures 2.0 would provide transitional coverage of breakthrough devices that are deemed reasonable and necessary. *Cure SMA supports provisions that will increase access to breakthrough devices or treatments that a person with SMA may need to help maintain or improve their health and independence.*



- **Sec. 409: Medicaid electronic visit verification systems**: Cures 2.0 would prohibit the use of geographic tracking features and biometrics within state electronic visit verification (EVV) systems that were required under the 21st Century Cures Act. Many individuals with SMA who utilize Medicaid home and community-based services (which are covered under the EVV mandate) expressed great concern over their privacy due to EVV systems that could track their whereabouts. *Cure SMA supports the prohibition of collecting geographic or biometric information within state EVV systems.*

Thank you for the opportunity to provide comment on the 21st Century Cures 2.0 discussion draft. For more information, your staff can contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

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