



Make today a breakthrough.

July 25, 2022

Dear Member of Congress,

Cure SMA is pleased to share key findings of a recent report that shows considerable progress has been made in support of individuals with spinal muscular atrophy (SMA), thanks in part to past congressional policies and investments. As we approach August's SMA Awareness Month, we also highlight the need for continued research in SMA to address significant unmet needs of individuals with SMA.

The [State of SMA](#) report analyzed data from Cure SMA databases to provide the first-ever comprehensive examination of the status of SMA, a neuromuscular disease that impacts the muscles used for activities such as breathing, eating, crawling, and walking.

The report found that:

- ***SMA treatments are reducing SMA mortality and helping individuals with SMA to meet key developmental milestone.*** ⁱ[\[Page 24\]](#)
- ***Adults with SMA (18+ years) represent the largest segment of the U.S. population with SMA,*** which was historically the leading genetic cause of infant death.ⁱⁱ [\[Page 10\]](#)
- ***Newborn screening of SMA is saving lives and decreasing the time to diagnosis and treatment.***ⁱⁱⁱ [\[Page 19\]](#)

Past congressional investments, policies, and federal programs have contributed toward the exciting progress made in the last decade in SMA. However, individuals with SMA and their families continue to report health care needs and structural and societal barriers that impede their ability to live independently and contribute toward their community.

During this anniversary of the Americans with Disabilities Act and in honor of next month's SMA Awareness Month, Cure SMA asks that you continue to address the needs and priorities of children and adults with SMA by supporting additional SMA research at the National Institutes of Health. Please support the SMA report language included in the House FY 2023 Labor-HHS-Education bill and the SMA community's [Greater Independence Agenda](#).

Thank you for your support of issues impacting children and adults with SMA.

Sincerely,

A handwritten signature in black ink that reads "K. A. Hobby".

Kenneth Hobby
President
Cure SMA

ⁱ SMA treatments are helping to reduce SMA mortality, which is down one-third since 2009 and change the course of the disease, with most (54%) individuals with SMA Type 1 (which was historically characterized by their lack of sitting independently) now meeting that key developmental milestone.

ⁱⁱ We estimate that the largest segment of the US population with SMA are adults (18+ years). For those which we have data for (n=276), most are employed or are seeking employment (69% work part- or full-time and 8% seek employment) and are highly educated (64% have a bachelor's degree or higher).

ⁱⁱⁱ Newborn screening of SMA has been implemented at a faster rate than other rare conditions, and in our databases, individuals identified via newborn screening report a median age of diagnosis confirmation of 7 days (n=52), while a subset of individuals that were not identified via newborn screening reported a median age of diagnosis of 96.5 days (n=80). Newborn screening is the most effective and efficient way for babies with SMA to access timely treatments and available support. Overall, time to diagnosis has decreased by 40 percent over the past decade.