Make today a breakthrough.

April 27, 2022

Re: NIH Report Language Request for your Labor-HHS-Education Appropriations Submission

Dear Senator,

Cure SMA respectfully asks that you support the unmet needs of children and adults living with a neuromuscular disease known as spinal muscular atrophy (SMA) **by requesting report language in your Labor-HHS-Education Appropriations Member Submission Form to continue National Institutes of Health (NIH) research into SMA and related nervous system disorders**.

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 cannot properly function and eventually die, taking away an individual's ability to walk, eat, and breathe. Historically, most babies born with SMA Type 1, the most common and severe form of the disease, died before reaching their second birthday, making SMA the leading genetic cause of infant death.[i]

The future for SMA is improving thanks to past investments Congress has made into the NIH. As highlighted in the FY 2023 congressional justification for the National Institute of Neurological Disorders and Stroke (NINDS), past NIH research has *"contributed to new treatments for rare disorders, including the first gene based, disease-modifying therapies for* ***spinal muscular atrophy*** *and muscular dystrophy."*[ii] Current SMA treatments can slow or stop future degenerative nerve damage. If delivered early, before the onset of symptoms, these treatments can greatly improve motor and developmental gains in individuals with SMA and lead to reduced future need for intensive health care and specialized supports, such as ventilators. In addition, past NIH-funded research into SMA has yielded new understanding of the nervous system and disease mechanisms that benefit other neurological and neuro-muscular diseases, a priority research area for the NIH.[iii]

However, current SMA treatments do not cure the disease or its debilitating symptoms. Significant unmet needs remain across all ages and disease stages of SMA. Individuals with SMA, particularly adults, the largest segment of the SMA population, reported ongoing challenges related to muscle weakness (90%) and fatigue (52%), due to degeneration that occurred prior to treatment.[iv] These struggles greatly impact their independence and ability to work and live independently in their communities. Even those individuals who received treatment before the onset of clinical symptoms may also display unmet needs, such as bulbar impairment and gait abnormalities. Their needs are also common across other neurodegenerative diseases, making continued NIH research into this cross-cutting area consistent with the 2021 law (P.L. 117-79) to *"advance the understanding of neurodegenerative diseases"* through a Public-Private Partnership for Neurodegenerative Diseases.[v]
Cure SMA asks that you help address the significant unmet needs faced by individuals with SMA by including the following report language in your FY 2023 Member Submission Form to the U.S. Senate Labor-HHS-Education Appropriations Subcommittee.

LABOR-HHS-EDUCATION APPROPRIATIONS SUBMISSION FORM INFORMATION

Appropriations Bill:	Labor-HHS-Education
Federal Department:	U.S. Department of Health and Human Services
Agency/Account:	National Institutes of Health (Office of the Director)
FY 2023 Cure SMA Request:	Report Language (see below)

Cure SMA Report Language Request:

Spinal Muscular Atrophy.—The Committee commends NIH for its past research into spinal muscular atrophy (SMA) that has led to new therapies to treat SMA and also contributed toward greater knowledge and research capacity into nervous system disorders. While current SMA treatments can slow or stop future degenerative nerve damage, they are not cures and there remains significant unmet need across all ages and disease stages of SMA. Individuals with SMA, particularly adults, the largest segment of the SMA population, face significant challenges in muscle weakness and fatigue due to degeneration that occurred prior to treatment. Individuals treated prior to clinical symptoms onset may also display unmet needs, such as bulbar impairment and gait abnormalities. The Committee urges NIH to address these unmet needs, which are common across other neurological and neuro-muscular diseases, by supporting new research into the role and function of survival motor neuron (SMN) protein, investigation into non-SMN pathways and targets capable of modifying disease, and research into how to best combine SMN-enhancing and non-SMN approaches for optimal therapeutic outcomes.

Thank you for your consideration of Cure SMA's request on behalf of individuals with SMA and their families across the country. For more information related to our request, 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,

Kenneth Hobby
President

Mary Schroth, M.D
Chief Medical Director

Jacqueline Glascock, PhD
Director, Research Programs

[i] Johns Hopkins Medicine, About SMA, 2022, https://www.hopkinsmedicine.org/health/conditions-and-diseases/spinal-muscular-atrophy-sma
[ii] National Institute of Neurological Disorders and Stroke, FY 2023 Congressional Justification (Page NINDS-18); https://www.ninds.nih.gov/sites/default/files/ninds_fy_2023_cj_chapter_508c_0.pdf
[iii] National Institute of Neurological Disorders and Stroke, FY 2023 Congressional Justification (Page NINDS-18); https://www.ninds.nih.gov/sites/default/files/ninds_fy_2023_cj_chapter_508c_0.pdf
[iv] Unmet Needs of the SMA Community, Cure SMA Survey, https://www.curesma.org/published-sma-research/
[v] Public Law 117-79, December 23, 2021, https://www.congress.gov/117/plaws/publ79/PLAW-117publ79.pdf