November 5, 2025

The Honorable Bill Cassidy
Chairman
Health, Education, Labor and Pensions Committee
United State Senate
Washington, DC 20510

The Honorable Bernie Sanders
Ranking Member
Health, Education, Labor and Pensions Committee
United States Senate
Washington, DC 20510

Dear Chairman Cassidy and Ranking Member Sanders:

Thank you for spotlighting the urgent health needs of Americans at your recent hearing on U.S. biotech manufacturing. Cure SMA represents individuals and families affected by spinal muscular atrophy (SMA), a rare neuromuscular disease. The SMA community is directly impacted by both the promise of biomedical innovation and the limitations of the current U.S. regulatory system. We are sharing the SMA community's perspective to urge the committee to act with urgency to reduce barriers to approval and access to lifesaving treatments and cures.

SMA causes irreversible nerve damage and severe muscle loss, impairing a person's ability to walk, breathe, and perform daily activities. The disease affects children and adults in all 50 states. Cure SMA is the national organization that supports individuals and families impacted by SMA through research, advocacy, and healthcare education.

The SMA community has benefited from research investments and policies that incentivized the development of treatments for rare diseases. To date, the U.S. Food and Drug Administration has approved three disease-modifying treatments for SMA. These treatments slow future disease progression and, when administered before symptom onset, can provide dramatic benefits. Before treatments were available, children born with the most severe form of SMA often did not live beyond their second birthday.[i] Today, many children born with SMA are thriving, thanks to early diagnosis through newborn screening and timely access to treatment. However, many older individuals with SMA face significant barriers to accessing existing treatments and related healthcare services.[ii]

Despite these biomedical advances, substantial unmet needs remain. Most children and adults with SMA developed debilitating symptoms before treatments became available and therefore do not experience the full impact of these therapies. Current treatments do not regenerate nerves, reverse muscle weakness, or restore lost motor function. The SMA community urgently seeks new treatments to address ongoing health challenges such as muscle weakness, severe fatigue, and impaired motor and respiratory function.[iii] Unfortunately, recent regulatory decisions have slowed progress toward these goals.[iv]

Cure SMA supports the committee's review and oversight of the regulatory framework to eliminate unnecessary bottlenecks and inefficiencies highlighted at the hearing that delay or restrict access to promising therapies. We appreciated the sentiment expressed during the hearing that regulators should act with the same urgency as a parent of a child with a rare or chronic condition. The SMA community, which still faces significant disease-related

complications, needs that same level of urgency from policymakers. We urge the committee to continue its oversight and pursue legislative actions that ensure the U.S. biotech industry remains globally competitive, not only for its U.S. economic impact, but most importantly for its role in developing lifesaving treatments and cures. For people with progressive diseases like SMA, every day counts.

Thank you for holding a hearing and for considering the views of Cure SMA and the SMA community. Should you or your staff have any questions or need additional information, please contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202-871-8004 or maynard.friesz@curesma.org.

Sincerely,

Kenneth Hobby
President

Maynard Friesz
Vice President of Policy

Cc: Members of the Senate Health, Education, Labor and Pension Committee

[i] National Institutes of Health, https://pmc.ncbi.nlm.nih.gov/articles/PMC5776712/
[ii] State of SMA Report, Cure SMA, https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf#page=29
[iii] State of SMA Report, Cure SMA, https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf#page=34
[iv] Cure SMA Treatment Delays, https://www.curesma.org/sma-treatment-delays-faq/