



Make today a breakthrough.

April 26, 2024

Re: Please Sign onto Newborn Screening Funding Dear Colleague

Dear Senator

On behalf of individuals and families impacted by spinal muscular atrophy (SMA), **Cure SMA respectfully asks that you sign onto the Dear Colleague letter circulating by Senators Gillibrand and Lankford in support of fiscal year 2025 funding for newborn screening activities at the U.S. Department of Health and Human Services (HHS).** The state newborn screening programs funded through HHS were critical in helping the SMA community achieve universal newborn screening in all 50 states.

SMA is a neuromuscular disease that destroys the muscles used for everyday living activities such as walking, swallowing, and breathing. Before treatments, babies born with SMA Type 1, the most common form of the disease, often died before reaching their second birthday, making SMA the leading genetic cause of infant death.

Since 2016, the U.S. Food and Drug Administration (FDA) has approved three SMA treatments that— if delivered early, before the onset of SMA symptoms—are making it possible for babies born with SMA to thrive and meet key developmental milestones. New data shows that 100 percent of newborns with 2 SMN2 copies who received treatment within two weeks of birth are walking at age two whereas zero percent walk when they receive a treatment after 90 days. The keys to this success are an early diagnosis and early access to an SMA treatment.

Newborn screening ensures that families of babies born with SMA receive an early diagnosis and can make timely decisions about treatment and care. In 2018, the federal newborn screening advisory committee added SMA to the federal recommended uniform screening panel (RUSP) and recommended that all 50 states begin screening for SMA. In January 2024, the SMA community achieved its goal of universal newborn screening when we reached SMA implementation in all 50 states. This major accomplishment means that families of babies born with SMA today will no longer experience the frustration of delayed diagnosis and missed opportunities for better outcomes.

Federal newborn screening activities highlighted in the Gillibrand-Lankford Dear Colleague letter help support states in their effort to implement newborn screening. This funding was key in helping the SMA community achieve its goal of newborn screening of SMA in all 50 states. The HHS resources fund all 50 state newborn screening programs to help them add and test conditions, such as SMA, to their newborn screening program. In addition, the funding helps support the federal advisory committee, which makes state screening recommendations.

Even though the SMA community has already achieved newborn screening in all 50 states, we fully support funding newborn screening programs and activities to help ensure that state programs have the resources and technical expertise they need to meet the needs of their



residents and their families. **Please consider signing onto the Gillbrand-Lankford Dear Colleague letter in support of newborn screening funds by contacting Laura Bain (Sen. Gillibrand) at Laura_Bain@gillibrand.senate.gov or Cambridge Neal (Sen. Lankford) at Cambridge_Neal@lankford.senate.gov.**

Please don't hesitate to contact Cure SMA should you have any questions or need further information, your staff contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202-871-8004 or maynard.friesz@curesma.org.

Sincerely,

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

Kenneth Hobby
President

A handwritten signature in black ink that reads "Maynard Friesz".

Maynard Friesz
Vice President of Policy