ABOUT SMA AND CURE SMA

Spinal muscular atrophy (SMA) – the number one genetic cause of death for infants – robs people of physical strength by affecting the motor nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe. The disease is caused by a mutation in the survival motor neuron gene 1 (SMN1). Without enough of the SMN protein, nerve cells cannot function properly and eventually die, leading to debilitating and often fatal muscle weakness.

Cure SMA is the largest network of families, clinicians, and research scientists working together to advance SMA research, support affected individuals/caregivers, and educate the public and professional communities about SMA.

HAWAII CHAPTER INFORMATION

Cure SMA has 36 volunteer-led chapters across the United States. To find and contact the Hawaii chapter, visit www.curesma.org/chapters

SMA CARE CENTER NETWORK

SMA Care Center Network is the centerpiece of our efforts to address the changing landscape of SMA. The goal of the SMA Care Center Network is to develop an evidence-based standard of care that will improve the lives of all those affected by SMA.

Care Center located in Hawaii:
Currently there are no Care Centers in Hawaii at this time.

NEW HOPE FOR TREATING SMA

Thanks to the dedication of our community and the ingenuity of our researchers, we now have treatments that target the underlying genetics of SMA. Currently, there are two treatments for SMA approved by the U.S. Food and Drug Administration (FDA) – Spinraza and Zolgensma. Both are SMN-enhancing treatments.

But our work is not done. We know what needs to be done to develop and deliver effective therapies that target other systems, pathways, and processes affected by SMA. Our goal is a combination of therapeutic approaches that can be tailored to each individual’s age, stage, and type of SMA. These breakthroughs will continue to change the course of SMA for everyone affected—from infants to adults—and eventually lead to a cure.
NEWBORN SCREENINGS FOR SMA IN HAWAII CAN SAVE AND IMPROVE LIVES

Early diagnosis and treatment of spinal muscular atrophy (SMA) can lead to improved, long-lasting developmental outcomes for individuals living with SMA. In addition, clinical data shows that SMA treatments and care are more effective when delivered early and pre-symmetrically. Newborn screening is the most effective and efficient way for babies with SMA to access timely treatments and available supports.

SMA INCLUDED ON NATIONAL RECOMMENDED NEWBORN SCREENING PRIORITY LIST

In July 2018, the U.S. Secretary of Health and Human Services added SMA to the national recommended list for newborn screening—known as the Recommended Uniform Screening Panel or RUSP.

Each state determines what conditions to include in its screening panel, and how to add conditions to this panel. The RUSP is an important guideline for the states in this process, and after being included, several states have taken action to adopt and implement newborn screenings of SMA.

HAWAII NEWBORNS NOT CURRENTLY SCREENED FOR SMA

Hawaii has not adopted or implemented statewide newborn screenings for SMA.

PROCESS TO GET SMA ADDED:

The Department of Health organizes a task force for the condition they are considering to review. They then review the evidence for that condition and make a recommendation to the State NBS Advisory Committee. Then the committee makes a recommendation whether to approve the condition to the Director of Health. The Director of Health will then task the NBS program to add the condition if he/she accepts the recommendation of the committee.

HAWAII FAMILIES IMPACTED BY SMA SEEK ACTION ON NEWBORN SCREENINGS

Hawaii residents and families impacted by SMA, supported by Cure SMA, seek immediate action on:

- Adoption and implementation of statewide newborn screening for SMA; and
- Support for federal funding for rare disease basic research and newborn screening activities.

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