CURE SMA CARE CENTER NETWORK

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

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 multiple treatments for SMA approved by the U.S. Food and Drug Administration (FDA)—Evrysdi, Spinraza, and Zolgensma. All 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 impacted 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 impacted—from infants to adults—and eventually lead to a cure.

NORTH CAROLINA CHAPTER INFORMATION

Cure SMA has 36 volunteer-led chapters across the U.S. To find and contact the North Carolina chapter, visit www.curesma.org/chapters.

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Care Center located in North Carolina:
- Duke University Medical Center, Durham, NC

TYPE 1 SMA
Onset: Before 6 months
Milestones: No sitting

TYPE 2 SMA
Onset: 6 - 18 months
Milestones: Sitting, not walking

TYPE 3 SMA
Onset: Childhood after 12 months
Milestones: Walking

TYPE 4 SMA
Onset: After 30 years old
Milestones: Normal

NORTH CAROLINA ESTIMATES

North Carolina

Est. individuals living with SMA: 378
Est. babies born with SMA annually: 11
Est. number of SMA carriers: 207,672

Estimates for incidence, prevalence, and carriers are based on 2018 birth and population data for the state of North Carolina.
Early diagnosis and treatment for 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 delivered early and especially when pre-symptomatic. Newborn screening is the most effective and efficient way for babies with SMA to access timely treatments and available supports.

NEWBORN SCREENINGS FOR SMA IN NORTH CAROLINA CAN SAVE AND IMPROVE LIVES

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 for SMA.

Cure SMA has made implementation of universal screening for SMA—as recommended by the federal government—a top priority. Thanks to the advocacy of individuals and families impacted by SMA and the leadership of state officials, well-over half of all states have implemented newborn screening for SMA, representing nearly 7 in every 10 babies born in this country.

Despite the progress in screening newborns for SMA, the U.S. remains well short of the goal of 100 percent universal newborn screening for SMA. Several states are still not screening babies born in their state for the leading genetic cause of death among infants.

All babies born in North Carolina are screened for SMA through a state pilot program! Cure SMA applauds North Carolina for being an early adopter of newborn screening for SMA. We encourage the state to fully implement this measure as soon as possible so North Carolina parents of newborns with SMA have the information they need to make timely decisions about care and treatment for their child.

For more information, contact the Cure SMA Advocacy Team at advocacy@curesma.org

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