**SMA STATE FACT SHEET**

**Nebraska**

Est. individuals living with SMA: 81  
Est. babies born with SMA annually: 2  
Est. number of SMA carriers: 38,585

Estimates for incidence, prevalence, and carriers are based on 2018 birth and population data for the state of Nebraska.

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**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.

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**NEBRASKA CHAPTER INFORMATION**

Cure SMA has 36 volunteer-led chapters across the United States. To find and contact the Nebraska chapter, visit [www.curesma.org/chapters](http://www.curesma.org/chapters)

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**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 Nebraska:**

Currently there are no Care Centers in Nebraska at this time.

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**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.

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**TYPES OF SMA**

There are four primary types of SMA that are based on the age of onset and highest physical milestone achieved. Type 1 is the most severe and most common, affecting 60 percent of those with SMA and is typically diagnosed during an infant’s first six months.

**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

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Last revised on November 2019
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-symptomatically. 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.

NEBRASKA NEWBORNS NOT CURRENTLY SCREENED FOR SMA

Nebraska added SMA to its newborn screening panel. However, the state has not yet implemented state-wide newborn screenings for SMA.

PROCESS TO GET SMA ADDED:

The Newborn Screening Advisory Committee evaluates the Evidence Based Review from the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC). The committee evaluates what resources are necessary in Nebraska to be able to implement screening for any new condition. They then make their recommendation. The Advisory Committee does not have authority to decide, only to recommend. Their recommendation is forwarded through the chain of command in the Department of Health and Human Services and if it is recommended to add the condition, the Department leadership decides if they can move forward with proposing regulation revisions. Any regulation revision proposed needs approval of the Governor’s Policy Research Office before it can go through the public hearing approval process. Alternatively, a Senator can introduce legislation to revise their governing statute by adding the condition there. Depending on how the statutory language reads, they may also need to go through regulation revisions if the statute passes. Either way they strongly request advocates to include the program and the Advisory Committee in whatever efforts they make.

NEBRASKA FAMILIES IMPACTED BY SMA SEEK FINAL ACTION ON NEWBORN SCREENINGS

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

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