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.
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 especially when pre-symptomatic. 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 for SMA.

CURE SMA’S GOAL: UNIVERSAL SCREENING 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.

THANK YOU, MISSOURI!

All babies born in Missouri are screened for SMA! Cure SMA applauds Missouri for being an early adopter of newborn screening for SMA. The state started screening newborns for SMA in 2019. Missouri parents of newborns with SMA now have the information they need to make timely decisions about care and treatment for their child.