WHAT ARE THE TREATMENTS, AND WHERE DO I GET THEM?

The first treatment for SMA, SPINRAZA, was approved in late 2016, and more treatments are being tested in clinical trials. Contact Cure SMA for more information, and make an appointment with your doctor to discuss the best approach to treatment for your baby.

WHAT SHOULD I DO NOW?

Your doctor will refer you to a specialist, who will order more tests to confirm whether your baby has SMA. The specialist may also order additional tests to determine the best approach to your baby's care, which could include starting medicine right away or carefully monitoring your child's condition for a time.

Either way, it's critical to have a healthcare provider begin watching your baby's development now. Your child may have the best chance at good health if treatment starts even before there are any signs of muscle weakness from SMA.

TAKE THE FOLLOWING ACTIONS RIGHT AWAY:

Contact your baby's doctor or other healthcare provider, and share these test results if you have not already. Say it's our baby immediately.

Ask your healthcare provider for a referral to a specialist. Often, though not always, you will be referred to a pediatric neurologist, a doctor who specializes in nerve diseases in children.

Get more information for you and for your baby's doctor or healthcare provider.

Free information is available from Cure SMA. Call Cure SMA at 800.886.1762, or email info@curesma.org.

RESOURCES

Cure SMA is a nonprofit patient advocacy group that provides information and support to parents and other caregivers.

cure SMA

Make today a breakthrough.

SMA

Spinal Muscular Atrophy

WHAT YOU NEED TO KNOW

About your newborn screening test results

www.cureSMA.org

WHY AM I RECEIVING THESE TEST RESULTS?

When your baby was born, blood was taken to test for conditions that could affect your child's health. The results of these newborn screening tests show that your baby likely has a condition called spinal muscular atrophy, or SMA.

There are ways to treat SMA. Your baby was tested at birth because it's important to start treatment early. Though your baby may be healthy now, infants with SMA have a missing or faulty gene that can cause serious health problems if not treated. A healthcare provider's early determination about treatment offers the best chance that your baby will stay as healthy as possible.

WHAT IS SMA?

SMA affects the nerves in the spinal cord that send signals to the muscles to tell them how to work. When these nerves don't work, muscles can't do their job and become very weak. People with SMA may have difficulty walking, eating, and even breathing because of this muscle weakness.

The condition is serious and can cause early death. But research shows that early treatment can help.

HOW DO I KNOW IF MY BABY IS DEFINITELY SICK?

The results of your newborn screening test show that your baby most likely has SMA. Your doctor may order more testing to confirm the diagnosis. Additional testing may also provide information that helps predict how serious a case of SMA your child has.

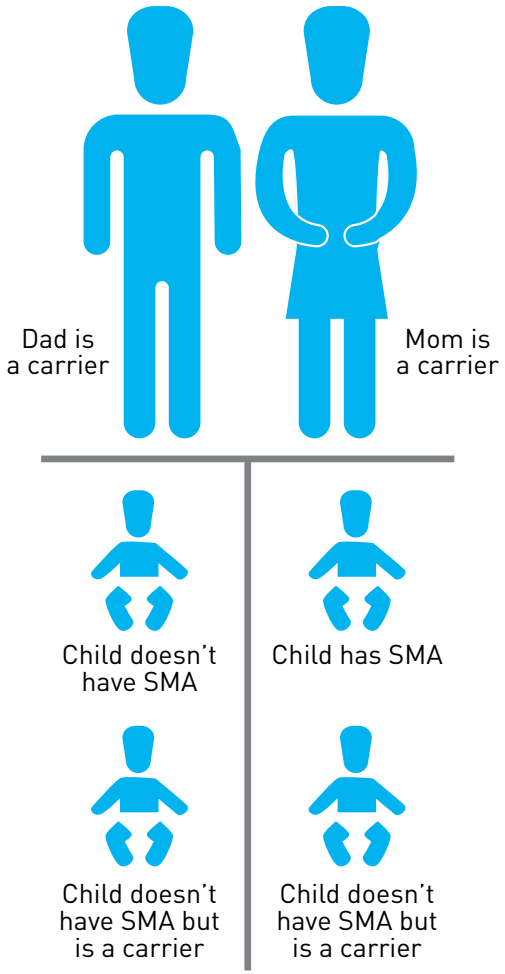
When you will visibly see signs of SMA depends on the type of SMA your baby has. Most cases of SMA are type I, which is the most serious, with symptoms appearing within 6 months of birth. In less common, milder cases, symptoms may not appear until later in life.

Depending on how serious your child's condition is, the best chance to prevent dangerous muscle weakness in your child is to get treatment early.

I DON'T KNOW ANYONE WITH THIS DISEASE. HOW COULD MY BABY HAVE IT?

SMA is caused by a missing or faulty gene known as the SMN1 gene. Babies usually receive two copies of this gene—one each from the mother and father. Because you need only one functioning SMN1 gene to be healthy, parents may pass down a missing or faulty SMN1 copy without knowing it. A baby born with SMA received missing or faulty SMN1 gene copies from **both** parents.

The figure below shows the chances that a healthy mother and father who are SMA carriers—each with one working SMN1 gene and one missing or faulty SMN1 gene—will have a child with SMA. In each pregnancy, the chance of these parents having a child with SMA is 1 in 4, or 25%.



NEWBORN SCREENING REGISTRY

The Cure SMA Newborn Screening Registry NBSR (NBSR) is an online Registry established to help our SMA community (including affected individuals, families, clinicians and researchers) learn more about SMA, better manage symptoms over time, and develop new treatments.

We invite you to participate by going to the NBSR website and following the instructions to provide Cure SMA with information about your child.

The NBSR is a program of Cure SMA. Cure SMA is the sole guardian of NBSR and its material. NBSR information can be used to improve clinical care and to support new therapy development. Registries in other diseases also have a long history of success in movig research and clinical care forward.

To access the NBSR portal click here_____(will insert URL to portal) to receive additional information or to register your child or patient.

This brochure contains important information about your baby's newborn screening test. It's critical to your baby's health to talk to a healthcare provider about possible treatment right away.

We know this information may come as a surprise. We're here to help. We are a nonprofit advocacy group that focuses specifically on spinal muscular atrophy, or SMA, the condition identified on your baby's screening test. Contact us for information, guidance, or support.

Phone: 800.886.1762
Email: info@curesma.org