

TALKING POINTS FOR CHARITY STREAMERS

GENERAL INFORMATION

WHAT IS SPINAL MUSCULAR ATROPHY (SMA)?

Basic: Spinal muscular atrophy (SMA) is a progressive neurodegenerative disease that impacts the muscles used for activities such as breathing, eating, crawling, and walking.

Detailed: Spinal muscular atrophy (SMA) is a progressive neurodegenerative disease that affects the motor nerve cells in the spinal cord. Because muscles in the body can't respond to signals from these damaged motor nerves, they will weaken and shrink. This can impact the body's ability to breathe, eat, crawl, and walk.

WHAT IS CURE SMA?

Basic: Cure SMA is a nonprofit organization dedicated to the treatment and cure of spinal muscular atrophy (SMA). We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide individuals with SMA and their families the support they need for today. Learn more at www.curesma.org.

Detailed: Cure SMA is a nonprofit organization dedicated to the treatment and cure of spinal muscular atrophy (SMA). Since 1984, Cure SMA has grown to be the largest U.S.-based network of individuals, families, clinicians, and research scientists working together to support individuals and families impacted by SMA, advance SMA research, and educate public and professional communities about SMA. Cure SMA funds and directs comprehensive research that drives breakthroughs in treatment, advances access to high-quality care, provides practical support programs, and advocates for the needs of the SMA community. Learn more at www.curesma.org.

FAST FACTS

- SMA impacts the ability to breathe, eat, crawl, and walk
- SMA does not affect a person's ability to think, learn, and build relationships with others
- Each year, thousands of infants in the U.S. and around the world are born with SMA
- Approximately 1 in 50 people, more than six million total, is a genetic carrier for SMA
- SMA and carrier status can be detected through a simple genetic blood test
- As of January 2024, all 50 states are screening newborns for SMA
- Cure SMA provided basic research funding that led to the first ever FDA approved treatment for SMA
- Cure SMA has invested more than \$82 million in research and have funded half of all the ongoing new drug programs for SMA
- While there are several approved treatments, we do not yet have a cure for SMA

TALKING POINTS

CURE SMA IS COMMITTED TO CHANGING THE LIVES OF PEOPLE WITH SMA

We are the largest network of families, healthcare providers, and research scientists working together to support individuals with SMA, advance SMA research, and educate public and professional communities about SMA. We work on a national, local, and personal level to ensure that our SMA community will receive the greatest impact possible from donor contributions.

CURE SMA IS A VALUABLE RESOURCE FOR THE SMA COMMUNITY

A diagnosis of SMA can be overwhelming, and Cure SMA is often the first place that families and individuals will turn to for answers and support. In addition to driving critical scientific and medical advances, Cure SMA works to improve quality of life for people with SMA and their families. We help people understand what the changing landscape of SMA means and offer the information and resources they need to live independent, successful, and fulfilling lives.

CURE SMA HELPS EMPOWER THE SMA COMMUNITY THROUGH EFFECTIVE ADVOCACY

We believe the voice of our community is powerful. That's why we work directly with legislators, regulators, and other officials to advocate on behalf of the SMA community. We also give our community the resources and opportunities they need to advocate for themselves.

CURE SMA AND ITS ADVOCATES HAVE ENSURED THAT 99% OF BABIES BORN IN THE U.S. ARE NOW SCREENED FOR SMA AT BIRTH.

Evidence shows that early diagnosis through newborn screening (NBS) enables children to reach their full potential. Early detection and timely administration of approved SMA therapies can prevent the rapid and irreversible loss of motor function caused by SMA. The U.S. has 49 states that currently screen newborns for SMA.

WITH ADVANCES IN TREATMENT, CARE FOR THOSE WITH SMA IS MORE IMPORTANT THAN EVER BEFORE.

Treatments are extending lifespan, meaning the number of individuals living with SMA will increase. Cure SMA works with healthcare providers to ensure that every individual has access to a physician who understands SMA and can explain treatment and care options.

CURE SMA IS VITAL TO ADVANCING RESEARCH

For nearly 40 years, Cure SMA has invested in and led the research that has made today's breakthroughs possible. With connections and expertise in both the patient and research communities, we're uniquely positioned to direct funds to where they can have the most impact. We have invested more than \$82 million in research and have funded half of all the ongoing new drug programs for SMA.