



www.stopsma.org



## What is Spinal Muscular Atrophy?

- ◆ SMA is the number one genetic killer of young children.
- ◆ It causes severe weakness in muscles from head to toe.
- ◆ Weakness worsens as the child grows.
- ◆ It causes weak pulmonary function, low lung capacity.
- ◆ Diagnosis means the child is missing a gene that produces protein that keeps motor neurons healthy.
- ◆ Motor neurons are needed so your brain can communicate with your muscles.
- ◆ Moderate cases cause children to lose weight bearing strength in their legs which bounds them to a wheelchair. They also have weak arms, torsos and necks.
- ◆ Severe cases cause children to lose the ability to swallow, talk, make facial expressions and/or breath on their own.
- ◆ It is often called a childhood version of ALS (Lou Gherig's Disease).
- ◆ **There is no treatment... yet.**

## Why there's hope for an SMA treatment

- ◆ Researchers know the cause of SMA: a certain missing gene. This puts SMA ahead of many genetic and neuromuscular disorders.
- ◆ Treatments are being developed for the cause not just the symptoms.
- ◆ Miraculously, the missing gene has backup/twin genes in the body. These genes can be targeted with a treatment. Research has shown that these genes can be activated to produce the missing protein.
- ◆ The number of backup genes a child has determines the severity.
- ◆ Gene therapy is being researched to activate the backup genes and/or replace the missing gene altogether.
- ◆ Therapies are being developed from many different theories, targeting genes, muscles, and motor neurons.
- ◆ Ten years ago there was only one research project designed specifically for SMA treatment
- ◆ Five years ago there were only four research projects designed specifically for SMA treatment
- ◆ **Today there are 15 research projects designed specifically for SMA treatment** and three of them are in patient clinical trial.
- ◆ More trials and research programs are coming.
- ◆ The National Institute of Health described SMA as the neuromuscular disease closest to a cure.

**You can help by donating or sponsoring our events.**