

Fast Facts: SMA

SMA is common.

1 in 10,000 infants has SMA.

SMA is aggressive.

Children with SMA suffer from progressive weakness and lack of muscle control.

SMA is deadly.

It is the leading genetic killer in children under age 2.

SMA may be treatable.

New therapy is on the horizon, and with your support, we can give these children hope.



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Spinal Muscular Atrophy



Finding a Cure

What is SMA?

Spinal Muscular Atrophy (SMA) is a genetic disease that destroys motor neurons in the spinal cord.

Over time, children with SMA lose muscle control and strength, which leads to the progressive inability to walk, stand, sit up, eat, and breathe.

There is currently no cure for SMA, but medical treatment can help prevent respiratory and nutritional problems. The prognosis for survival beyond the age of 2 depends on the age of onset and the rate of progression of the disease.

Understanding Genetics

Almost all children with SMA are missing a functioning copy of a gene called SMN1, which is crucial in producing a protein needed for motor neurons to survive. They do have a back-up SMN2 gene, but it does not work properly. Early research showed that some drugs, such as hydroxyurea, may improve expression of SMN.

Clinical Trials at Stanford University Medical Center

Ching Wang, MD, PhD, pediatric neurologist at Lucile Packard Children's Hospital at Stanford, is at the forefront of SMA research and treatment. Dr. Wang is conducting FDA-approved clinical trials using the drug hydroxyurea to boost a child's SMN2 gene so that it can generate more functional, neuron-strengthening protein.

A Reason to Hope

When motor neurons are strong, the effects of SMA are not as severe – and children with this disease may live more normal lives. Dr. Wang's groundbreaking research has already enabled some children with SMA to regain voluntary muscle movement, and a few have even spoken for the first time.

"We are seeing amazing results that we didn't even dare dream of. It's not here yet, but with proper funding and research, I think we could be heading towards a cure."

– Ching Wang, MD, PhD

This is one-of-a-kind SMA research that is currently only taking place at Lucile Packard Children's Hospital and Stanford University Medical Center.



SMA patient, Mathieu,
with Ching Wang, MD, PhD

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accelerate this
life-changing research.**

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