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.

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