CHILDREN'S® PROJECT

ATAXIA-TELANGIECTASIA

"ay-TACK-see-uh teh-LAN-jick-TAY-sha"

Children with ataxia-telangiectasia (A-T) are born perfect and seemingly healthy – with limitless potential – until compromises are forced on them by this debilitating, deadly disease. By supporting and coordinating aggressive research, we hope to give them a chance to live and enjoy life's possibilities.

MEET RHETT!

Rhett is 5 and he is the sweetest little human. Our story started when he was around the age of 2. Up until that point he had been growing normally and meeting his milestones. We started noticing however that he was more "wobbly" and "slobbery" than the other kids his age. He was clumsier on his feet. Some of his exam at times began to mimic Cerebral Palsy but his story didn't fit. His pediatrician initially set us up with physical therapy and speech therapy for evaluation. We were also seeing ENT and Pulmonary for his recurrent upper respiratory infections. After a few sessions with physical therapy, we knew we needed to see Neurology. Blood work then led to concerns for Muscular Dystrophy. We were all over the diagnosis map and so were our emotions. Labs, labs, more labs, and an MRI later we still had no idea what we were dealing with. Nothing seemed to fit. Frustrated, we then saw a neuromuscular specialist and different Neurologist who immediately sent us to Genetics. "This is not Cerebral Palsy." "This is not Muscular Dystrophy." Genetics started with a simple panel of genes to rule out muscle disorders. When those results were inconclusive yet again, we broadened out the genetic panel to examine his full genome. Two years later with this DNA test, we found our defective ATM genes. Our biggest fears have come true that we are dealing with something that has no cure, no treatment, and is progressive. After receiving this heartbreaking diagnosis, I cannot begin to tell you how thankful we are for the A-T Children's Project (A-TCP). Community is everything and this foundation has set us up with others in the United States and around the world that share our same story. The A-TCP is working vigorously with researchers around the globe to find a cure for ataxia - telangiectasia (A-T). So we are now on mission, and we are racing the clock. Thank YOU for helping us RACE to a cure! Your support is the hope we need and that these kids deserve! Your donations and time WILL make a difference. Now let's rock, roll, and STOMP out A-T!

KIDS. HOPE. CURE.

ABOUT A-T

ATAXIA-TELANGIECTASIA (A-T) . . .

- is a rare genetic disease
- attacks in early childhood
- progressively affects coordination
- predisposes patients to fatal cancers
- severely compromises the immune system

CHILDREN WITH A-T ARE . . .

- as rare as 1 in 40,000 births
- from all races and ethnicities
- usually unable to walk by age 10
- may not survive their teens and rarely survive their twenties

Carriers of mutated copies of the A-T gene are approximately 1 per 100 individuals in the general population.

Compared to the typical population, the cancer rate of children with A-T is 1,000 times higher, and the cancer rate of carriers can be up to 4 times higher.

AREAS OF RESEARCH FUNDED BY THE A-T CHILDREN'S PROJECT

- Establishing and supporting the Global A-T Family Data Platform, a direct patient engagement website and data donation platform
- Spearheading and recruiting for clinical trials, including antisense oligonucleotide (ASO) gene therapy
- Discovering how the A-T protein works in brain cells to reveal ways to compensate or correct for the lack of the protein in children with A-T
- Screening thousands of compounds to find potential new drugs to help A-T
- Neuroimaging to reveal abnormal brain function in A-T
- Developing rating scales to measure A-T, helping power efficient clinical trials
- Developing human cell and animal models for A-T to aid drug discovery and development
- Conducting studies to improve the quality of care for people with A-T at the A-T Clinical Center at Johns Hopkins Hospital

Ataxia-telangiectasia (A-T) is a genetic disease that causes loss of muscle control and balance, cancer, lung disease and immune system problems in children and young adults, shortening their lives.

The nonprofit A-T Children's Project partners with academic and industry investigators worldwide – organizing and supporting innovative research, conferences, clinical teams, data platforms and biomarkers – to optimize disease management strategies, develop new treatments and find a cure.

MORE INFO: **Other ways to help:**

Host an event | Donate stock
Run a race | Make a monthly gift
Designate United Way donations
Spread the word

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