

#### Parents Leading Rare-Disease Drug Development

"Yes, They Can!" is a parent-led campaign to fund the science to bring promising and groundbreaking gene therapy treatments to children with FOXGI syndrome.

### WHAT IS FOXG1 SYNDROME?

It's a rare children's disease resulting from a spontaneous mutation of the FOXG1 gene, one of the earliest and most critical genes in brain development. FOXG1 is an autism-related gene and linked to many other neurological disorders.

#### A FOXG1 LIFE IS HARD.





~50 seizures a week



tures Frequent hospital



~9 disability/ medical devices



[INSERT VIDEO]

~10 medications



24-hour care



~6 medical

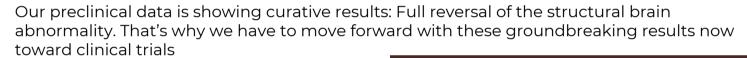
Most kids with FOXG1 syndrome can't talk, walk, sit up or take care of their basic needs. Most suffer from seizures and respiratory failure, need feeding tubes and undergo multiple surgeries. FOXG1 syndrome has been linked to autism, Alzheimer's, brain cancer, epilepsy and more.

WE'RE CHANGING HOW DRUGS FOR RARE DISEASES ARE DEVELOPED.

# THE FOXG1 CENTER OF EXCELLENCE IS ALREADY ACHIEVING GROUNDBREAKING CURATIVE RESULTS!

FOXG1 Research Foundation can move more quickly than academia and drug companies. Since 2017, we have:

- Discovered that the FOXG1 gene persists after birth.
  This discovery is critical to fixing FOXG1 syndrome in humans.
- **Produced** groundbreaking curative preclinical gene therapy results.
- **Funded** 11 academic labs and moved from basic to translational science.
- Developed more than 20 models for testing new therapies
- Established the first and only FOXG1 Patient Registry, an invaluable research database.
- Pioneered the Digital Natural History Study model.
- Became a Chan Zuckerberg Initiative Rare as One Partner and Mentor.
- Hosted 3 science symposiums and the first-ever FOXG1 parents conference.



## WE NEED YOUR HELP.

The goal of this campaign is to take the next step toward bringing our gene therapy to clinical trials.

The next step on the roadmap to successful clinical trials is Toxicology. This is where we study the safety and identify potential adverse effects of our treatment.

We need \$5 million to get this done.



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