



# The TSC Alliance launches the 50 FORWARD FUND



The TSC Alliance improves quality of life for everyone affected by TSC by catalyzing new treatments, **driving research toward a cure** and expanding access to lifelong support. We are striving to create a future where everyone affected by TSC can live their fullest lives.

The **50 Forward Fund** is the TSC Alliance's largest research initiative in our 50-year history. It was designed as part of the TSC Alliance's Research Business Plan to guide research efforts and develop evidence-based standards of care for TSC individuals. The **50 Forward Fund** bolsters the momentum we've built since our founding while ambitiously looking forward to a future where everyone affected by TSC can live their fullest lives.



Lesley and Alex celebrate every milestone in the life of their daughter, one of the first ever to be saved by medication *in utero*. Read their story at [tscalliance.org/50ForwardFund](https://tscalliance.org/50ForwardFund).



Shelly and Matt have two children with TSC 12 years apart. Those 12 years of TSC research have defined their journeys in profoundly different ways. Read their story at [tscalliance.org/50ForwardFund](https://tscalliance.org/50ForwardFund).

Amaan wasn't supposed to walk at all, let alone across the stage at his high school graduation. TSC research changed the course of his life. Read his story at [tscalliance.org/50ForwardFund](https://tscalliance.org/50ForwardFund).



## Through the 50 Forward Fund, we will advance research toward the following goals:

- Focusing research to better understand TSC and identify new treatments
- Altering the course of the disease and improving quality of life through prenatal diagnoses and preventative treatments
- Educating medical professionals to achieve more rapid diagnosis and better treatment aligned with evidence-based standards of care

To achieve these ambitious research goals, we're counting on continued investment, collaboration and unwavering commitment of our community. We expect the following research outcomes to make a meaningful, positive impact on the lives of all those touched by TSC.

- Pilot newborn screening test for TSC.
- Publish updated guidelines for unaddressed aspects of TSC care, including reproductive and perinatal health and SUDEP.
- Require a transition-of-care plan at all TSC Clinics.
- Create a TSC-Associated Neuropsychiatric Disorders (TAND) Patient-Reported Outcomes (PRO) tool to be used in clinical trials that measures changes in the most challenging TAND symptoms for each individual.
- Move at least two projects from preclinical testing into clinical trials.

# OUR PLAN FOR ACCELERATING THE NEXT GENERATION OF TSC RESEARCH AND DISCOVERY

We believe the next breakthroughs in TSC research are on the horizon—and to achieve the progress our community is depending on, we're investing \$40 million in four key areas of research.

TSC affects everyone differently. To understand which drugs will be most effective for each individual, researchers need biosamples, clinical data and patient-reported outcomes. These require efficient collection processes, standardized analysis protocols and robust data management.

Natural History Database and Biosample Repository  
**\$11 Million**

Clinical Research  
**\$8 Million**

Clinical studies require extensive planning and collaboration. The TSC Alliance aims to alleviate the obstacles that stand in the way of efficient, effective and safe clinical trials.

The Preclinical Consortium fills a critical research gap for standardized, rigorous testing of potential new TSC therapies prior to clinical trials. This innovative platform provides industry and academic members access to reproducible preclinical models and outcome measures, providing an unbiased platform for this critical stage in drug development. The Preclinical Consortium removes risks associated with developing new drugs and will encourage more companies to focus on treatments for our community.

Preclinical Consortium  
**\$11 Million**

Innovative Research  
**\$10 Million**

Our goal is to award more research grants annually, forge stronger connections between the top researchers around the world and bring stakeholders together at Innovation Workshops.

“Understanding TSC echoes into other disorders like epilepsy, autism, cancer. So, by focusing on this rare disease we are learning a lot about some common diseases as well. It is a very vibrant research environment.”

Peter B. Crino, MD, PhD, Chair of Neurology, University of Maryland School of Medicine



“Only through discovery can we determine what the best practices and therapeutics might be and learn how to take better care of kids and adults with TSC.”

Shafali Spurling Jeste, MD, Chief, Division of Neurology at Children's Hospital Los Angeles



## THE LEADERS TAKING US INTO THE FUTURE OF TSC RESEARCH

### Steve Roberds, PhD

Steve joined the TSC Alliance staff in 2011 as Chief Scientific Officer, after spending 16 years in the pharmaceutical industry. In this role, he leads the development and execution of the TSC Alliance's research strategy through partnerships and conversations with all stakeholders, including individuals and families affected by TSC, basic and clinical researchers in academia and industry, healthcare providers, government agencies involved in medical research, and other non-profit organizations.



### Dean Aguiar, PhD

Dean joined the TSC Alliance in November 2018 with more than 17 years of research and development (R&D) leadership in biopharmaceutical and medical device industries, leading teams and technologies from discovery to investigational new drug and investigational device exemption, a pre-requisite for clinical trial evaluation. He brings an entrepreneurial and collaborative approach to R&D, identifying opportunity, defining strategy and developing a scientific data package to warrant clinical translation of technologies to benefit the patient, the family and the care provider.



### Chip and Kristin Burkhalter

Chip and Kristin are parents to Bear, age 10, with TSC as well as two other boys. The impact of TSC on their family has led them to be passionate advocates for all those who are affected by TSC. They believe strongly in the impact TSC research will have on Bear's future, as well as the future of all those living with TSC. They devote their time and energy as chairs of the **50 Forward Fund**, knowing the more we learn about TSC, the better chance we have at funding new treatments for some of the most challenging TSC manifestations.





# The TSC Alliance launches the 50 FORWARD FUND



## WE NEED YOU TO JOIN US

Contribute to the **50 Forward Fund** today and help us reach forward-thinking milestones on our way to life-changing research results.

The **50 Forward Fund** will make a lasting impact on the lives of people affected by TSC. Donate now!

**Invest in the 50 Forward Fund and make a real difference in the lives of people affected by TSC. DONATE NOW!**

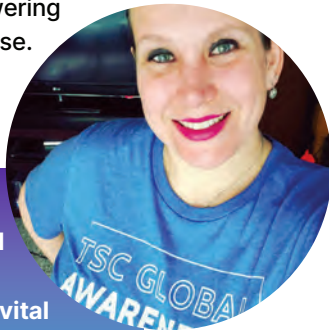


**“I believe it should be everyone’s mission to support research, because it is with science that the most important discoveries are made.”**

Anita Bhatia, MHA, CEO & Executive Director, Ramesh and Kalpana Bhatia Family Foundation

## Additional ways to help

- Host a fundraising event to gather resources that could change the course of TSC.
- Introduce the TSC Alliance to people in your community who care about discovering new therapies for epilepsy, autism or cancer.
- Put the TSC Alliance in touch with private foundations or donor advised funds interested in medical research or empowering people living with a rare disease.



**“My life has been enriched because of recent discoveries. Research is vital to prolonging lives so I hope with more funding, we can unlock the answers we have so desperately been seeking.”**

Jennifer Waldron, TSC adult



**“One of the things that impresses me about the TSC Alliance is their ability to identify research needs and collaborate effectively with researchers. Donation to this organization means they can get results faster.”**

Lorne Waxlax, 50 Forward Fund Donor

## CONNECT WITH US



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## WHY THE TSC ALLIANCE

### About TSC

Tuberous sclerosis complex (TSC) is a rare genetic disease that causes tumors to form in many different organs, including the brain, eyes, heart, kidneys, skin and lungs. Worldwide, TSC affects nearly 1 million people and approximately 50,000 people in the United States.

Every individual's experience with TSC is different—many are able to live independently despite the disease, while others experience more challenges requiring complex care. Changes in the brain caused by TSC have the biggest impact on quality of life, from seizures and developmental delays to intellectual disabilities, behavioral challenges and autism.

TSC is a linchpin disease, meaning its genetic pathway also plays a role in other diseases and disorders. Advancements in TSC research may lead to a better understanding of autism, epilepsy, traumatic brain injury, diabetes and cancer—diseases that affect more than 65 million people in the United States alone.

### The TSC Alliance is here for everyone affected by TSC

The TSC Alliance improves quality of life for everyone affected by TSC by catalyzing new treatments, **driving research toward a cure** and expanding access to lifelong support.

Founded in 1974 by four mothers of children with TSC, the TSC Alliance quickly became the leading source of support and hope for those affected by the disease. Since then, we have made incredible progress in raising awareness, accelerating research discoveries and creating support systems around the world.

Today, the TSC Alliance is a leader in the rare disease community and a comprehensive resource for anyone touched by TSC. We won't stop until every person with TSC can realize their fullest potential—no matter how complex the journey is to get there.

### We have a proven track record of success accelerating research.

The TSC Alliance is committed to further catalyzing research to create meaningful outcomes for people living with TSC and their families. Since 1984, we have funded more than \$37 million in TSC research and leveraged \$505 million in additional federal funding.

Our accomplishments to date include:

Backing the first-ever preventative clinical trial for epilepsy in the United States

Creating a Biosample Repository with more than 2,650 samples linked to clinical data in the Natural History Database

Funding basic research that ultimately contributed to FDA approval of three drugs specifically for TSC

Building a Preclinical Consortium to help move new treatment ideas toward clinical trials

“The ongoing efforts into TSC research provide a strong sense of hope as we collectively move forward to change the course of the disease.”

Tanjala Gipson, MD, TAND Unit  
Director at Tuberous Sclerosis  
Center of Excellence at Le Bonheur  
Children's Hospital

