



TANGO2RESEARCH
FOUNDATION

**ANNUAL
REPORT
2023**

COLE AND KAITLYN

WWW.TANGO2RESEARCH.ORG

FOUNDER'S MESSAGE



Mike Morris
President & Founder



Kasha Morris
Secretary & Founder

Dear Friends & Supporters,

On behalf of the board of directors, foundation staff, and volunteers, we are proud to share some of the highlights and successes of the TANGO2 Research Foundation over the past year.

The most compelling narrative continues to be the B-vitamin story and the many firsthand accounts shared by parents and caregivers on how transformative this has been to patient health and well-being. It is especially rewarding to know that it was the combined efforts of families, researchers, and clinicians supported by the foundation that facilitated this breakthrough.

There is always more to learn, of course, and the foundation awarded an additional \$110,000 USD in research grants in 2023 to bring our total investment since 2018 to \$760,000 USD. We expect that total to near one million dollars as we move through 2024 and continue to aggressively support new projects and initiatives.

Since our inception, we've been a patient-centered organization. We were very fortunate this past year to receive \$250,000 USD in funding from the Patient-Centered Outcomes Research Institute (PCORI) to establish a learning network that accelerates and expands our capacity around patient and family involvement in rare disease research. This is a two-year project that brings together a broad coalition of stakeholders across our community. This grant will also make it possible for us to host our third in-person TANGO2 Family Conference in June of this year.

In closing, we continue to be thankful to a core group of researchers and clinicians who freely collaborate with one another and give selflessly of their time to further the understanding around TANGO2 Deficiency Disorder (TDD). We're optimistic that their work, along with the collective efforts of families and all those involved with the foundation, will make 2024 an amazing year of breakthroughs and insights for the TANGO2 community.

With Gratitude,

Mike & Kasha Morris

OUR MISSION

TO LEAD THE WAY IN FINDING A CURE FOR TANGO2 DEFICIENCY DISORDER

OUR VISION

TO ACCELERATE THE UNDERSTANDING OF TANGO2 BY ADVOCATING AND PROMOTING AWARENESS, SUPPORTING AND CONNECTING FAMILIES & DRIVING EARLY AND ACCURATE DIAGNOSIS

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OSIRIS AND ORION

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GREAT LAKES / SOUTHEAST -
AMY CLAY
ROBIN MITCHELL

USA - TEXAS / CENTRAL -
MARICELA LOPEZ

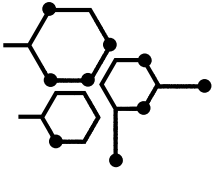
22Q11.2 COMMUNITY -
DEBBIE DELOACH



ARTHUR & EMIL

OUR COMMITTEES

OUR IMPACT



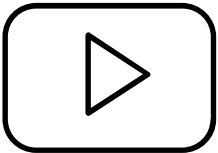
THE FOUNDATION AWARDED \$110,000 IN RESEARCH GRANT DOLLARS TO STUDY TANGO2 DEFICIENCY DISORDER TOTALING \$760,000 SINCE 2018



HELD TANGO2 TALK THAT ALLOWED FAMILIES TO LEARN ABOUT DIET AND NUTRITION FOR TANGO2 DEFICIENCY DISORDER AFFECTED INDIVIDUALS



RECRUITED NEW MEMBERS TO THE FOUNDATION'S SCIENTIFIC ADVISORY BOARD AND BOARD OF DIRECTORS TO FURTHER OUR MISSION



RECEIVED 126,700 VIEWS ON OUR YOUTUBE CHANNEL, IMPROVING EDUCATION AND RAISING AWARENESS ABOUT TANGO2 DEFICIENCY DISORDER



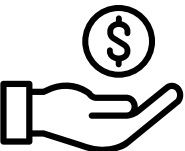
AWARDED FUNDING FROM; PATIENT CENTERED OUTCOMES RESEARCH INSTITUTE (PCORI), UPLIFTING ATHLETES, HORIZON THERAPEUTICS/AMGEN, & EVERY LIFE FOUNDATION



HELD A RESEARCH SYMPOSIUM WITH OVER 70 ATTENDEES THAT SHARED THE LATEST TANGO2 RESEARCH & BROADENED OUR RESEARCH NETWORK



UNEARTHED SCIENTIFIC BREAKTHROUGHS OF B-VITAMINS FOR INDIVIDUALS LIVING WITH TANGO2 DEFICIENCY DISORDER FUELING UPDATED CLINICAL RECOMMENDATIONS PUBLISHED IN GENEREVIEWS



RAISED \$111,000 WITH OUR COMMUNITY-WIDE SHINE A LIGHT ON TANGO2 CAMPAIGN & \$55,000 WITH OUR TANGO2 BENEFIT BANQUET IN CONNECTICUT



RECIPIENT OF A \$250,000 EUGENE WASHINGTON ENGAGEMENT AWARD FROM PCORI ALLOWING US TO ENGAGE MANY MORE STAKEHOLDERS IN THE RESEARCH PROCESS

REVENUE



- \$ CAMPAIGN FUNDRAISING
- \$ INDIVIDUAL DONATIONS
- \$ GRANTS & CORPORATE DONATIONS
- \$ OTHER DONATIONS

EXPENDITURES



- \$ RESEARCH GRANTS & FEES
- \$ FUNDRAISING
- \$ MANAGEMENT, SUPPORT & OPERATIONS
- \$ CONFERENCE & PROGRAMS

2022

102,955
161,676
183,394
4,098

452,124

2023

171,600
152,429
74,240
1,928

400,197

240,261
20,570
135,326
88,919

485,076

170,705
9,052
198,853
9,052

392,573

TREASURER'S MESSAGE



Mirroring the unwavering spirit of our children, our community showed exceptional determination once again. We are pleased to share another year of steadfast support from our donors, enabling us to serve our families and contribute to impactful research.

We expanded our Shine a Light on TANGO2 campaign by an impressive 25%, reaching more fundraisers and donors than ever before. Among our biggest drivers this year was the story of **the Fryar Family**. We united in support, remembering **Hudson** and celebrating **Cole's** remarkable resilience.

The foundation was successful in securing a little over \$280,000 (USD) in pledged grants and corporate donation support with the largest award being a two-year \$250,000 (USD) Eugene Washington Patient-Centered Outcomes Research Institute (PCORI) Engagement Award. Our mission, our progress, and every success story have been made possible because of our donors and supporters.

Kara Cameron



AISLYNN

OUR PROGRESS



The Foundation consistently arranges virtual gatherings, aptly named "TANGO2 Talks," designed to unite families impacted by TANGO2 deficiency disorder. These sessions play a crucial role in nurturing a strong sense of community and connection among participants. Within this supportive environment, attendees have the opportunity to receive the latest updates from the Foundation and stay well informed about ongoing initiatives.

In 2023, the Foundation invited **Brandy Rawls-Castillo**, a pediatric clinical dietitian, as part of its ongoing commitment to elevate the well-being of the TANGO2 disease community. This session centered around exploring optimal dietary strategies for maintaining children's health, placing special emphasis on the ongoing discourse regarding the benefits of B-vitamins—a subject underscored by various researchers during the preceding year's family conference.



2023 TANGO2

RESEARCH SYMPOSIUM

Led by leading TANGO2 deficiency disorder (TDD) researchers and healthcare professionals, the symposium provided a program of information-rich sessions spotlighting current research efforts propelling scientific advancements in TDD. We shared recent progress in TDD clinical care, including prospective recommendations for TDD therapeutic approaches, ensuring learners are on the cutting edge of TDD treatment and management. For those who may have missed it, visit our website or YouTube channel to view the recording.



MOHAMMED

OUR PROGRESS

EARLY DETECTION AND DIAGNOSIS



The diagnostic journey for families in the rare community is often lengthy and challenging, taking several years on average for an accurate diagnosis. Recognizing the critical need for expedited diagnosis, our Early Detection and Diagnostic Committee, established in August 2022, is dedicated to advocating for and engaging in activities that reduce the diagnostic odyssey. Committed to ensuring timely access to care, support, and treatment, this group actively works towards saving lives, improving quality of life, and raising awareness. Since its inception, the committee has achieved significant milestones, including advocating for the inclusion of TDD on gene panels, incorporating accurate language on various websites discussing TANGO2 deficiency disorder, co-authoring and branding a TDD resource for the Hispanic population in Mexico, expanding committee membership from 4 to 9 individuals, and participating in Global Genes' Rare Disease Mentoring program.



COMMUNITY-WIDE CAMPAIGN

The TANGO2 Research Foundation successfully concluded its third annual Shine a Light on TANGO2 fundraising campaign, dedicated to funding research for TANGO2 Deficiency Disorder (TDD). From February to June, participants formed fundraising teams, surpassing the \$100,000 (USD) goal with a total of over \$111,000 (USD) raised. Special recognition goes to top fundraisers Kara Cameron, Matt Dort, Veronica Jones, Team Ryan, and Team Fryar Brothers. These funds support T2RF Cycle 3 Research Grantees, advancing groundbreaking TANGO2-related research by scientists from Brigham and Women's Hospital, Baylor College of Medicine, and Concordia University. The foundation extends gratitude to all participants and looks forward to continued support. The celebration is available on our YouTube channel if you missed it.

BENEFIT BANQUET & AUCTION

On November 5th, **Kasha and Mike Morris**, parents of Ryan Morris, orchestrated the TANGO2 Benefit Banquet & Auction in Portland, Connecticut, with the primary objective of raising funds for TANGO2 deficiency disorder research. Themed "Around the World with TANGO2," the event featured speakers that described its global impact for families living in various regions. The evening seamlessly blended inspiration, information, and entertainment, incorporating live music, auctions, and a wine pull. The event's triumph, raising over \$55,000 (USD), can be attributed to the generous support of sponsors and the unwavering dedication of Kasha's good friends and family members who planned the event. These funds will contribute to advancing the foundation's mission of enhancing the lives of those affected by TANGO2 deficiency disorder. The event stands as a testament to the resilience of community, hope, and determination, reflecting a collective endeavor to forge a brighter future for individuals affected with this rare condition.



OUR PROGRESS

MOVING THE NEEDLE IN THERAPEUTICS

The power of patient voices emerged at the 2019 TANGO2 Family Conference, a year after the inception of the TANGO2 Research Foundation. Families participating in a Natural History Study (NHS) shared experiences, answering over 100 questions about their children to identify patterns related to TANGO2 Deficiency Disorder (TDD). Even with only 12 patients at that time, a pattern was emerging showing that kids who had never had a life-threatening crisis, were taking vitamins, particularly, B vitamins. By the year 2023, the evidence was reinforced with the participation of nearly 100 NHS contributors. Children taking B vitamins exhibit a reduced risk of metabolic crises, as indicated by the ongoing NHS. Notably, a publication from the NHS reveals that vitamin supplementation not only prevents crises but also enhances neurological outcomes. Additional findings highlight the positive impact of B vitamins on siblings and demonstrate the corrective effects of B5 on fruit flies with TDD symptoms. Furthermore, a study using TDD heart cells establishes the role of B9 in correcting heart arrhythmias.

Here are a few testimonials from TANGO2 families:

“The difference the new B vitamins have made for Phoebe is astronomical! She has gone from walking 50 meters to running all day. No wheelchair needed!”

“Sol has made great improvements since starting the vitamins. She has changed 100%. She does not need rest periods and has energy throughout the day.”

This is an example where a life saving drug supplement was found for a life limiting, newly discovered, little understood, rare disease because patient/families had a voice and a clinician/scientist listened. We

don't know yet what the function of TDD is or why the vitamins work. That will come. But for now, we celebrate that lives are being saved and quality of life is improving for TDD families around the world.

As we advance in researching the molecular and cellular events linked to TANGO2, the significance of the NHS will continue to grow. It will continue to serve as a conduit through which we can translate our discoveries into innovative clinical treatments, and ultimately a cure. For NHS participation and more information, visit the T2RF website to learn more.

Study Site: Baylor College of Medicine and Texas Children's Hospital, Houston Texas
Contacts: Drs. Christina Miyake & Seema Lalani

Email: tango2.research@bcm.edu or info@tango2research.org



JOHN & DR. LINA GONZALEZ

OUR PROGRESS

RESEARCH IS AT THE EPICENTER OF FINDING A CURE

We envision our foundation as the vehicle committed to driving the research forward: funding grants, enhancing communication and coordinating efforts among those invested in conquering TANGO2 deficiency disorder.

In 2023, we invested **\$110,000** in research around the world bringing the total planned investment to more than **\$760,000** since 2018.



COLLECTIVE RESEARCH IMPACT

18 Researchers



18 Projects



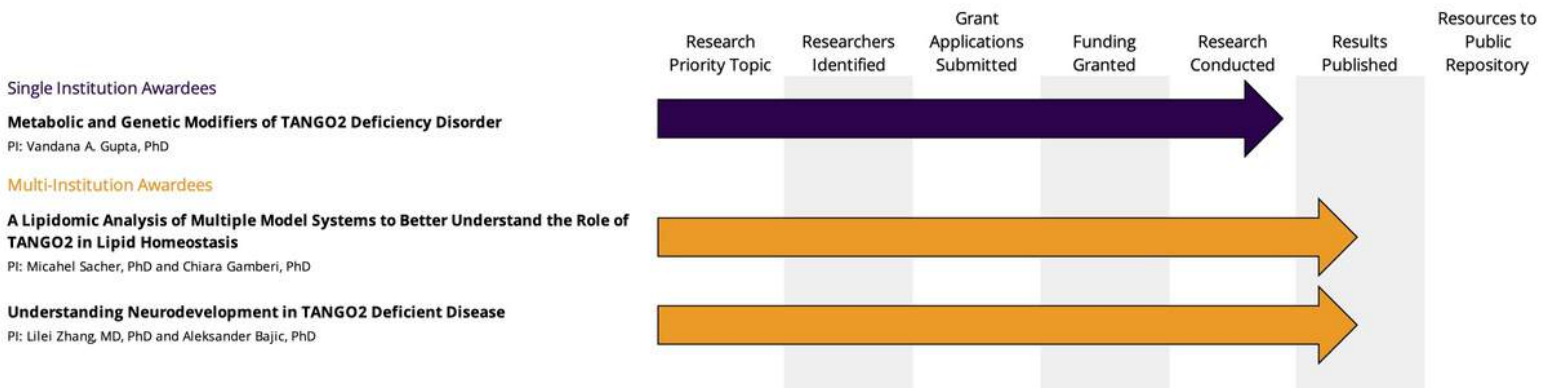
15 Institutions



5 Countries



CURRENTLY FUNDED RESEARCH



RESEARCHER'S CORNER: UPDATES ON PREVIOUSLY FUNDED T2RF RESEARCH

Project Name: Determine the Molecular Basis and the Metabolic Predisposition to TANGO2 Crises Using Human Induced Pluripotent Stem Cell Differentiated Cardiomyocytes

Project Principal Investigators: Christina Miyake, MD, MS & Lilei Zhang, MD, PhD

Funding Cycle: 2021 Open Call Project Duration: 2021 - 2023

Project's Results: Drs. Miyake and Zhang generated four heart cell lines from TANGO2 patients, including two with perfect matched controls. In addition, they established a distinct cell line for scientific rigor by editing cells from a healthy individual, removing TANGO2 to create a different model, and producing a perfect matched control. These lines, derived from induced "stem" cells, with the potential to become any cell type in our body, were shared with global researchers to facilitate diverse studies on TANGO2 deficient disorders. Funding for the creation of these cell lines supports the foundation's worldwide research efforts into understanding and studying this disease.



OUR RESEARCH

Using these heart cells, Drs. Miyake and Zhang conducted extensive studies on the cause and treatment of abnormal heart rhythms associated with TANGO2. These rhythms pose the highest risk for TANGO2 patients, with limited treatment options. Their research has identified a life-saving therapy—treatment with the B-vitamin folate effectively suppresses abnormal heart rhythms. Progress has been made in developing advanced technologies for studying these rhythms, with new results expected soon. Additionally, they've dedicated significant efforts to understanding the function of the TANGO2 protein, aiming to establish a unifying mechanism that explains all symptoms.

Visit our website to learn more about our foundation funded research and upcoming grant opportunities.

TDD IN FOCUS: SHARPENING THE LENS FOR STAKEHOLDERS TO ENGAGE IN PATIENT-CENTERED OUTCOMES RESEARCH (PCOR)

We were honored to receive a \$250,000 (USD) two-year Eugene Washington Engagement Award from the Patient-Centered Outcomes Research Institute (PCORI). PCORI, an independent nonprofit organization authorized by Congress, funds research that equips patients, caregivers, and clinicians with evidence-based information, a crucial resource for families impacted by rare diseases. The Eugene Washington PCORI Engagement Award Program aims to support projects fostering a community better prepared to actively participate in the research process. This aligns with our ongoing mission and will remain a key objective throughout the grant's lifecycle and beyond.

The funding from this award will drive the establishment of an international integrated stakeholder TDD Research Network, enhancing capacity for the TANGO2 Research Foundation's patient-centered, clinical effectiveness research (CER) initiatives. This will be achieved through the development and sustenance of a research-proficient community comprising family, researchers, and other stakeholders actively engaged in the research process from inception to completion.

During the fall of 2023, we carefully selected a twelve-person project stakeholder team including patients, families, researchers, and clinicians specializing in TDD. Additionally, we hired a research project coordinator, **Annie Kofoed**, to manage and support project activities as well as developed a rigorous and patient-centered agenda for our upcoming 2024 TANGO2 Family Conference.

As part of the capacity-building process, we are developing a TDD specific PCOR/CER-focused toolbox, workshops, forums, a research roadmap and an awareness campaign to guide stakeholders in conducting meaningful research projects for the TDD community.

Over the next two years, our foundation will remain committed to supporting TANGO2 families, raising awareness of the condition, advocating for earlier diagnosis, and advancing groundbreaking TDD research.

Special Acknowledgements

We would like to thank the 12 project stakeholders who are sacrificing their time and talents to sit on our project team:

Amy & Katie Clay, Cheyenne Beach, MD, Debbie DeLoach, Lina Gonzalez, MD, Dan Hernandez, Amanda Hull, Sam Mackenzie, MD, PHD, Josh Meisner, MD, PHD, Chaya Murali, MD, Ryan Carrier, MD, and Bob Mitchell.

We would also like to thank the following organizations for their collaboration and or support on this project: The Chan Zuckerberg Initiative Rare As One Network, Baylor College of Medicine/Texas Children's Hospital, UPMC, University of Rochester, Yale School of Medicine, The International 22q11.2 Foundation, and The National Organization for Rare Disorders.

This project was funded through a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI Engagement Award (30416).



LILIAN

OUR RESEARCH

ADVANCING TOMORROW'S DISCOVERIES: EMERGING RESEARCH INNOVATORS



From the Lilei Zhang Lab at Baylor College of Medicine, **Dr. Andrea Wilderman** will be presented with a \$20,000 Research Grant from Uplifting Athletes & the TANGO2 Research Foundation. As a member of Uplifting Athletes' 2024 Young Investigator Draft Class, Andrea Wilderman is among a group of 10 researchers who will each receive an unrestricted grant for medical research. Every researcher is nominated by a patient advocacy organization (PAO) recognized by Uplifting Athletes as a priority partner, with grants equally co-funded by Uplifting Athletes and the nominating PAO.

Dr. Wilderman completed her PhD in biomedical sciences at the University of Connecticut, Health Sciences. She recently joined the T2RF Early Detection & Diagnosis Committee.

As an active committee member, Andrea consistently devotes time to connect with individuals who are affected, their families, and patient advocacy organizations like ours, both within her home state of Texas and across a broader geographic scope. Her willingness to listen, empathize, and collaborate fosters a sense of belonging and hope among our members. Additionally, she joined the lab of Dr. Lilei Zhang, at Baylor College of Medicine to apply experimental and computational methods for the detection of genetic variants and epigenetic modifications related to congenital heart disease (CHD) and the use of adult stem cell models to identify effects on cellular function related to these genetic and epigenetic differences. Through this work, Dr. Wilderman aims to study genetic variation in regulatory elements associated with genes linked to CHD and novel therapeutic approaches for the treatment of rare diseases like TANGO2 Deficiency Disorder (TDD).

"I was introduced to the world of TANGO2 research by my thesis advisor and was touched by both the challenges faced by patients and families as well as the resilience of the TANGO2 community. It is deeply meaningful and profoundly humbling to do research that so directly impacts the lives of TANGO2 families. I am honored to contribute as best I can to finding answers for this community and hope for a cure."

- Sarah Sandkuhler



Sarah Sandkuhler, an Early Diagnosis & Detection committee member and recent recipient of a National Institutes of Health (NIH) Ruth L. Kirschstein National Research Service Award (NRSA) Individual Predoctoral Fellowship is a MD/PhD candidate at the University of Rochester School of Medicine and Dentistry. She graduated magna cum laude from the University of Rochester in 2019 with a B.S. in Neuroscience and is currently a student in the laboratory of Dr. Samuel Mackenzie. Her thesis work focuses on studying TANGO2 Deficiency Disorder in a *C. elegans* (worm) model system.

OUR COMMUNITY

WE'RE GROWING

As our research agenda grows, so does our need for support. Annie Kofoed joined our team as a Research Project Coordinator, leveraging her diverse talents, advocacy passion, and operational management expertise to advance the mission of the TANGO2 Research Foundation for resounding success.

Additionally, we welcomed Dr. Samuel Mackenzie as chair of the Scientific Advisory Board. Dr. Mackenzie is a researcher & pediatric neurologist at the University of Rochester. Since diagnosing his first patient in 2019, he has been committed to helping advance the standard-of-care for TDD and figuring out the function of the TANGO2 protein. Dr. Mackenzie & his team at Rochester are using human cell lines and a *C. elegans* (worm) model of TDD to study the effects of various stress conditions and how these improve with B-vitamins. He is also assisting with the ongoing Natural History Study, working with other pediatric neurologists at Rochester and Baylor to build a better understanding of movement disorders in TDD.



"I first got excited about this disease as a candidate for gene replacement therapy. It's humbling but also quite exciting to see how much vitamins have changed that picture. There may still be a role for gene replacement, but the idea of being able to give kids a supplement that keeps them out of the hospital is huge. I think we're going to see a very different treatment landscape in the next few years."

- Sam Mackenzie, MD, PhD



OUR COMMUNITY

DECODING MOVEMENT IN TANGO2 DEFICIENCY DISORDER

The true incidence of movement disorders in TDD remains unknown due to limited characterization in existing literature and our natural history study. Anecdotal reports mention undefined behavioral episodes marked by fatigue, neck tilt, drooling, dyscoordination, and weakness. These episodes, often prevalent early in life, pose diagnostic challenges. Even after a TDD diagnosis, unclear episodes with normal

laboratory studies create confusion for parents and care teams regarding appropriate treatment approaches.

Dr. Mackenzie and a team of movement experts assessed a library of videos from our families, identifying dystonia (sustained or intermittent muscle contractions causing abnormal, often repetitive, movements, postures, or both, often worsened by voluntary action and associated with overflow muscle activation) and ataxia (inability to generate a normal or expected voluntary movement trajectory that cannot be attributed to weakness or involuntary muscle activity about the affected joints) as common movement disorders in children with TDD. Ongoing family interviews aim to further characterize these movement disorders and "TANGO2 spells", including frequency, duration, age of onset, provoking and alleviating factors, and impact on quality of life. Accurate classification of TDD movement disorders holds the potential to enhance diagnosis rates, deepen our understanding of TDD's pathophysiology, and enhance preparedness for future clinical trials.

A movement disorder is defined as a neurological condition that results in increased (hyperkinetic) or decreased (hypokinetic) involuntary movement (phenomenology).

WE ARE PROUD TO HAVE THE SUPPORT OF THESE LEADING ORGANIZATIONS COMMITTED TO ADVANCING OUR MISSION.

EACH SPONSOR CREATES AN OPPORTUNITY FOR THE FOUNDATION TO FUND RESEARCH, FIND TREATMENTS AND SUPPORT OUR FAMILIES.



OUR SUPPORTERS



**WE EXPRESS OUR
GRATITUDE TO THOSE
WHO GENEROUSLY
CONTRIBUTED
DONATIONS IN 2023.**

\$5,000+

MIKE & MELISSA BALDA
DWIGHT & JODI BRAZAL
KARA CAMERON
MATT DORT
POCHETTINO FAMILY
MIKE & KASHA MORRIS
SEMPRA ENERGY (SWETEK FAMILY)
ANNA RUSTIONI

\$2,500+

KATHY BALDA
PATRICIA & KEVIN BURNHAM
THE CLAY FAMILY
ELLEN & MARTY DERRIG
NOAH GIBSON
AMANDA & DAN HULL
BETH & RICHARD KELPEN
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JUSTIN WAGNER

\$1,000+

TINA & JIM ADAMS
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FISHER FINANCIAL SERVICES
CAREY CALVIN GARLAND
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SERGIO

**OUR
DONORS**

EXECUTIVE DIRECTOR'S MESSAGE

With overwhelming community backing, the TANGO2 Research Foundation accomplished record levels of volunteer engagement this past year. Every individual contributes significantly to funding research, supporting programs, and advancing research initiatives that prioritize the holistic well-being of our families—physically, mentally, and emotionally. As our community continues to thrive, we remain dedicated to addressing the evolving needs of our families.

Among our many efforts included enhancing leadership included recruiting a new member, **Thomas Kim** to our Board of Directors and reviving our Scientific Advisory Board. The pinnacle of our initiatives for the year was our year-end giving campaign, which featured personal narratives from our families and highlighted the proven benefits of B-vitamins. These stories underscored the transformative impact of our research and our commitment to finding treatments. Beyond that, it sparked hope and strengthened a sense of community, serving as an inspiration for the broader rare disease community.

Looking forward, we eagerly anticipate the 3rd TANGO2 Family Conference taking place this June in Orlando, Florida. This event will serve as a platform to explore the latest advancements and collaboratively shape the future of TANGO2 Research. We anticipate the largest turnout yet.

Another exciting development is for our community-wide fundraiser, Shine a Light on TANGO2. For 2024, we are thrilled to feature a family from the United Kingdom for the first time as our Ambassador Family— **The Driffill Family**. Throughout this campaign, Phoebe's story, along with her parents Hannah and Tim, and siblings Hope and Joseph, will be highlighted.




As we look forward to our fourth grant cycle, we are on the verge of surpassing a significant milestone—investing over 1 million dollars toward research. Our goal is to expand, grow, and achieve more progress than ever before. Your continued support is instrumental in driving our mission forward, and we are grateful for your passion, spirit, and dedication to this cause.



“Our unwavering dedication persists, propelling us forward in the relentless pursuit of progress. Together, we stand alongside our families, fostering a collective spirit that drives us to overcome challenges and embrace the possibilities of a brighter tomorrow.”

Ann Geffen



PHOEBE

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