



**ANNUAL
REPORT
2022**

JAKE

FOUNDER'S MESSAGE



Mike Morris
President & Founder

Kasha Morris
Secretary & Founder

Dear Friends and Supporters,

On behalf of the board of directors, foundation staff and volunteers, we are proud to share some of the TANGO2 Research Foundation's accomplishments this past year.

We held a transformative family conference to further unify our researchers and patient community.

We funded \$212,500 in new research projects. This brings our total research investment to \$632,000 across 12 research institutions and 5 countries since inception. We also hired a Research Engagement Director to further expand our network and accelerate these efforts.

We expanded our patient and family support offerings adding two countries to our Regional Support Program and created an Early Detection and Diagnosis committee to shorten the diagnostic odyssey for patients.

By continuing to work together in collaboration with the research community, and with the generous support of our friends, families and community, we are making a difference. Our collective story is one of hope, one that looks toward the future and a world free of TANGO2-related disease. None of this would be possible without all of you.

With Gratitude,

Mike & Kasha Morris

OUR GOAL

TO ACCELERATE THE UNDERSTANDING OF TANGO2 AND PAVE THE WAY TO FUTURE THERAPIES OF LOVED ONES

OUR MISSION

TO LEAD THE WAY IN FINDING A CURE FOR TANGO2-RELATED DISEASE

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OTIS

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EARLY DIAGNOSTIC & DETECTION

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RESEARCH

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OUTREACH

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PASCALE DANESI
SEEMA LALANI, MD



WE AWARDED \$212,500 IN RESEARCH GRANT DOLLARS TO STUDY TANGO2-RELATED DISEASE.



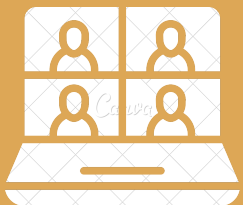
WE HELD A FAMILY CONFERENCE THAT ALLOWED FAMILIES AND MEDICAL PROFESSIONALS TO LEARN FROM ONE ANOTHER.



WE RECEIVED AN ADDITIONAL \$150,000 FROM THE CHAN ZUCKERBERG INITIATIVE AS A COHORT OF THEIR RARE AS ONE NETWORK.



WE ADDED 16 VIDEOS ON OUR YOUTUBE CHANNEL TO HELP EDUCATE AND SPREAD AWARENESS OF TANGO2-RELATED DISORDER.



WE ADDED AN EARLY DIAGNOSIS AND DETECTION COMMITTEE TO RAISE AWARENESS OF TANGO2-RELATED DISEASE AND INVESTIGATE METHODS OF EARLY DIAGNOSIS OF TANGO2 DISEASE.



WE ADDED REGIONAL COORDINATORS TO SUPPORT AND PROVIDE RESOURCES TO FAMILIES AROUND THE WORLD.



WE RAISED \$90,000 WITH OUR COMMUNITY-WIDE SHINE A LIGHT ON TANGO2 CAMPAIGN.



WE RECRUITED 95 PATIENTS TO JOIN THE NATURAL HISTORY STUDY ALLOWING US TO COLLECT INFORMATION TO BETTER UNDERSTAND TANGO2-RELATED DISEASE.



OUR IMPACT

© Mike Guggenmos Photography

REVENUE

\$ CAMPAIGN FUNDRAISING	79,782.76	102,955.31
\$ INDIVIDUAL DONATIONS	222,331.29	161,676.24
\$ GRANTS & CORPORATE DONATIONS	41,612.66	183,394.36
\$ OTHER DONATIONS	4,004.50	4,097.50
	<hr/>	<hr/>
	347,731.21	452,123.41

EXPENDITURES

\$ RESEARCH GRANTS & FEES	222,421	240,260.64
\$ FUNDRAISING	14,763.31	20,570.48
\$ MANAGEMENT & OPERATIONS	87,245.61	135,326.20
\$ CONFERENCE & PROGRAMS	11,218.76	88,919.08
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	335,648.68	485,076.40

TREASURER'S MESSAGE



Like the spirit of our children, our community proved to be resilient and determined. Despite being in a pandemic, we are proud to report another year of strong support from our amazing donors who enabled us to serve our families and fund more research than ever before.

We grew our Shine a Light on TANGO2 campaign by 10% and doubled our Giving Tuesday and Year-End Giving. Among our biggest drivers this year were the stories of **the Taggart family** and **the Mitchell Family**. These families inspired us to give more and do more.

As we look ahead toward growth and expansion of research activities with our new Research Engagement Director, **Deena Chisholm**, we remain committed to fiscal prudence to ensure sustainability for the families we serve.

Kara Cameron



KATE

OUR WORK

TANGO2 TALKS

Every quarter, our Foundation hosts virtual meetings for families called "TANGO2 Talks". Everyone impacted by TANGO2-related disorder is invited to come and have an opportunity to connect with others in the community.



These meetings provide community members with the most recent updates from the Foundation. Along with keeping the community up-to-speed on the work we are doing, we also engage our attendees in a variety of topics relevant to our international group.

This year, topics included research and Foundation updates, family-led Q&A with leading experts and special guests.



TANGO2 FAMILY CONFERENCE

This year's Conference brought our international TANGO2 community together in Orlando, Florida.

Over 2.5 days, attendees heard the latest on research and therapeutic development, care considerations, interacted with members of the community, and heard from the experts: TANGO2 patients and families.



For several families, the conference was the first opportunity to meet and engage with other affected families and TANGO2 researchers. Participation format was chosen to be as interactive as possible and designed to support learning, involvement, and sharing. Families got to speak directly with researchers, medical experts, caregivers and physicians. Participation and key learning from the conference directly influenced the future of research initiatives for TANGO2.

The TANGO2 Research Foundation is incredibly thankful to have received a grant from the Chan Zuckerberg Initiative as part of the Rare As One Cohort to host this conference for the TANGO2 disease community.

MEET DR. LINA GHALOUL GONZALEZ

Dr. Lina Gonzalez is a TANGO2 Research Foundation-funded researcher, a geneticist at UPMC Children's Hospital of Pittsburgh, and an assistant professor of pediatrics in the division of Genetic and Genomic Medicine at the University of Pittsburgh. Keep reading to learn more about her and why she is part of our community!

What made you want to get involved with TANGO2 research?

There is a lot that we don't know about this disease mechanism and treatment. Having a patient with this disease and knowing that I can contribute to the knowledge of the disease and ultimately treatment for many patients, makes this work rewarding.

What do you enjoy most about being a researcher for the TANGO2 Research Foundation?

I enjoy getting to know many people that *really* want to make a difference in this disease. This includes families of patients and many researchers from different institutions and countries. It is also amazing to see the drive behind the TANGO2 Research Foundation and its founders, Mike and Kasha Morris, and how much they achieved in such a short period of time.

What impact has the TANGO2 Research Foundation had on your life?

The TANGO2 Research Foundation supported me in doing my research for two years believing that my research can make a difference. In addition, through the Foundation, I was able to connect to many other researchers and clinicians working to try to understand TANGO2 deficiency.



SAMMY

About Dr. Gonzalez

Dr. Gonzalez received her degree in Medicine from the University of Aleppo School of Medicine in Aleppo, Syria. She is board certified in Internal Medicine, Medical Genetics and Genomics and Medical Biochemical Genetics. Dr. Gonzalez has been awarded a National Institutes of Health (NIH) K08 Mentored Clinical Scientist Research Career Development Award under National Human Genome Research Institute (NHGRI) in 2019 for her grant "Precision Genomic Medicine in The Plain Communities and its Impact on The Plain and General Population".

Dr. Gonzalez's focus is on the genetic disorder in the Plain people (Amish and Mennonites) in Western Pennsylvania to characterize novel genetic disorders or novel mutations. She also has interest in studying the pathophysiology of TANGO2 deficiency and identifying treatment targets.

Dr. Gonzalez loves spending time with her family (including her dog) and traveling, getting to know different cultures and connecting to people.

OUR COMMUNITY

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



MILENA

**Chan
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Initiative** 

UPMC
LIFE CHANGING MEDICINE

 **HORIZON.**

 **TOKIO MARINE
HCC**

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Therapeutics**

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Foundation**

**WE WOULD LIKE TO
THANK THE FOLLOWING
INDIVIDUALS FOR THEIR
DONATIONS IN 2022.**

\$5,000+

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POCHETTINO FAMILY
IKA SITORUS
JEFF WILLIAMS
MATTHEW DORT
MIKE & KASHA MORRIS
PETER MORRIS
SAMANTHA BURGESON
VERONICA & TYSON SWETEK

\$2,500+

AL-KARIM WALJI
ANDREA MURR
DAVE & KATHERINE AVERY
LAURA & BLAKE MOORE
LYNETTE ALLAN
PAT HUDDLESTON
PO FONG
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\$1,000+

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EDOARDO



**OUR
DONORS**

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-related disorder.

In 2022, we invested **\$212,500** in research around the world bringing the total planned investment to more than **\$632,000** since 2018.



OUR RESEARCH

COLLECTIVE RESEARCH IMPACT

18 Researchers



15 Projects



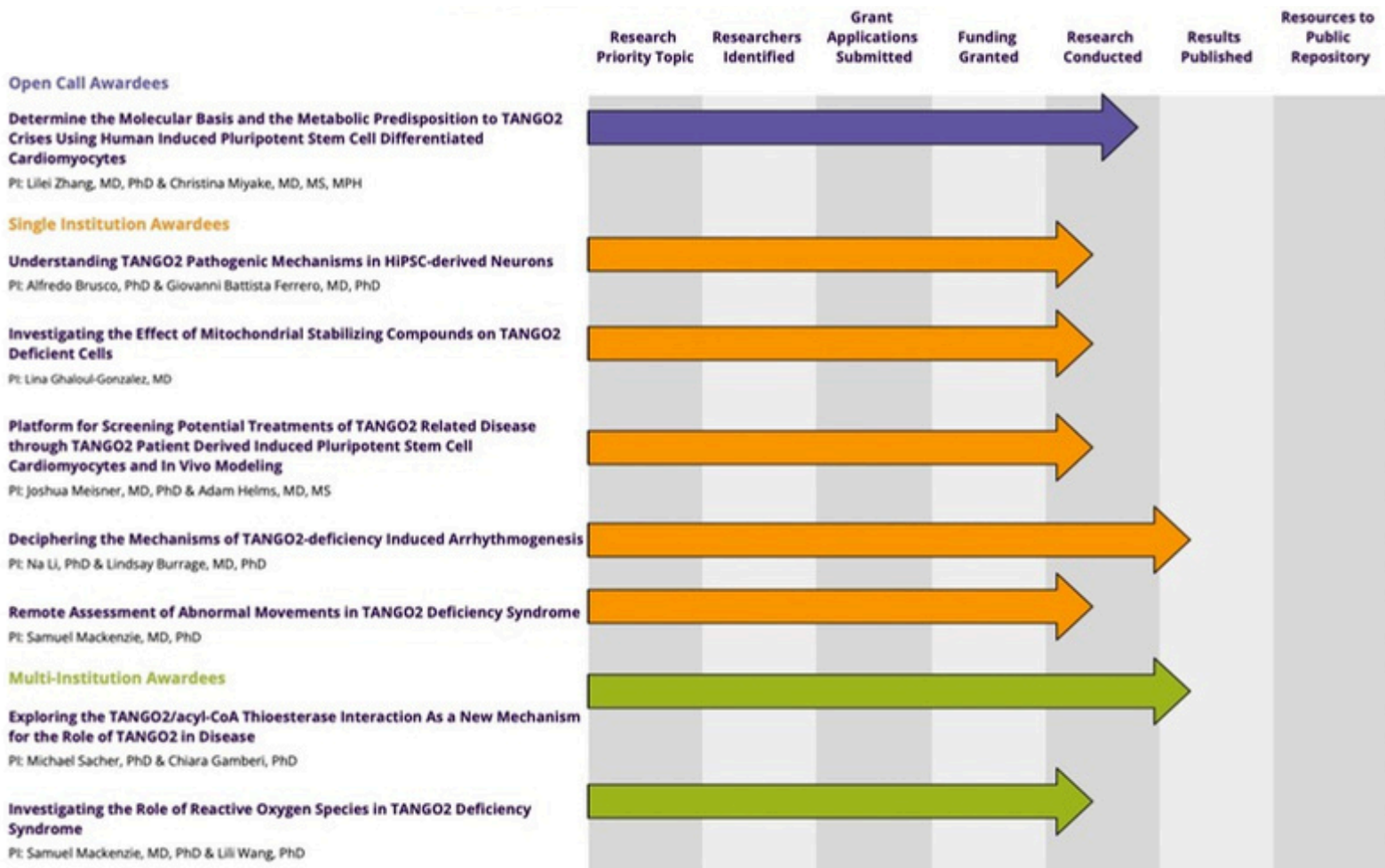
12 Institutions



5 Countries



CURRENTLY FUNDED RESEARCH



BRIA

OUR RESEARCH

RESEARCH PRIORITIES

Thank you to those who attended the family conference and completed the feedback survey at the conclusion of the event. From your responses, these topics are most important & will continue to help guide our research roadmap:

- Managing & understanding life-threatening symptoms of TANGO2-related disease
- Improving quality of life, independence and preventing loss of function
- Studying cellular response to medications
- Beginning efficacy clinical trials
- Discovering effective ways to avoid crises & rhabdomyolysis

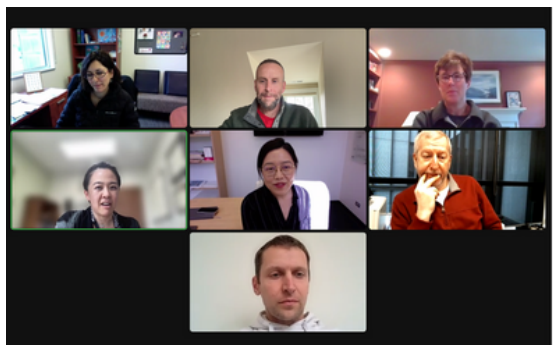
OPEN SCIENCE IN ACTION

In late February 2022, CZI opened a Request For Applications (RFA) for its inaugural Patient-Partnered Collaborations for Rare Neurodegenerative Disease. This presented the foundation with an opportunity to pursue to join the CZI Neurodegeneration Challenge Network. Although we did not receive this grant, the process proved to be a selfless commitment from our researchers working together in the spirit of open science can accelerate learning and maximizing impact.

What made this RFA unique is the requirement that a patient-led rare disease organization be at the center of the collaboration and be an active participant in the entire lifecycle. This flips the traditional model where institutions and researchers drive the process.

With this in mind, the Foundation set out to assemble an interdisciplinary team that could meet the challenge of this RFA and put forth a great application. The team consisted of Dr. Sam Mackenzie, Dr. Christina Miyake, Dr. Lilei Zhang, Dr. Michael Sacher, Dr. Lina Gonzalez and representing the foundation, Mike and Kasha Morris. The team had its first meeting in early March to begin the work of defining a project.

For most of the team members, it was the first time they had worked with one another and across multiple institutions with a patient-led organization in the middle. What started off as somewhat guarded conversations, quickly gave way to more open discussions and collaborations as a mutual trust was built across the group. By the fourth or fifth meeting, everyone had embraced the concept of open science with a shared sense of mission and purpose. Dr. Mackenzie was chosen to be the Principal Investigator (PI) on the RFA and led the team as it met about a dozen times between March and the May 2022.



What resulted was an incredibly strong application from an amazing and dedicated group of researchers. While the foundation was not ultimately awarded this competitive grant, this core group continues to meet on a monthly basis to share new findings and support one another's efforts moving forward.

WE'RE GROWING

As we continue to grow our research agenda so does the need for more support.

Deena Chisholm, MPH, CHES joined our team as the first Research Engagement Director to lead our research programs and stakeholders to participate in research vital to advancing treatments and a cure.

Additionally, we welcomed Dr. Joshua Meisner to the Foundation as the newest member of the Research Committee. Dr. Meisner is a researcher & pediatric cardiologist at the University of Michigan Congenital Heart Center. The TANGO2 disease patients he's cared for and their families have been an inspiration to him and sparked his interest in getting involved. Dr. Meisner is using tools in gene editing and cardiac muscle cell culture to understand the role of TANGO2 in the heart. His goal is to use these tools as an early test bed for potential treatments.



OUR RESEARCH



"I am hopeful that the combined efforts from our lab and across the TANGO2 research community will be able to translate our research findings into care for our patients in the clinic." - Josh Meisner, MD, PhD

MOVING THE NEEDLE IN THERAPEUTICS

The TANGO2 Foundation was founded first and foremost to advance research into treatment and a cure for TANGO2-related disease. The organization pursues this goal both by funding research and strategic investments that will speed scientific results into drug discovery.

A monoclonal antibody is a type of protein that is made in the laboratory and acts like human antibodies in the immune system.

To aid our researchers in their work with animal models, we hired ABclonal Technology to develop a monoclonal antibody for the TANGO2 protein. In the case of TANGO2, an antibody recognizing this protein will allow researchers to better understand where TANGO2 is found in the cell and what other proteins may be affected by its function. We are ecstatic to begin this new venture now. Scientific advancement brings incredible promise but also requires great patience. We are optimistic a better understanding of these aspects of TANGO2 will be a major step forward in understanding how mutations in this gene cause disease and in devising treatment options for affected individuals.

EXECUTIVE DIRECTOR'S MESSAGE

We wrapped up 2022 with several major projects to further enhance our presence, engage our community, and support families. We refreshed and redesigned our main website and fundraising website to better assist our community and establish our presence.

We also opened a third grant cycle to fund novel research seeking to advance the understanding of disease mechanisms and treatment of TANGO2 disease. We are grateful to our donors for the means to continue building our research network and fund great work.

Our volunteer base continues to grow as we add new members to our committees. We also formed a Clinical Advisory Board to develop and publish nutritional recommendations based on recent Natural History Study and research findings.

Our last effort of the year was the most successful Year-End Giving campaign we've ever had because of the Mitchell family. Ben Mitchell created a series of YouTube videos highlighting his sister, Hanna Mitchell's journey to diagnosis of TANGO2 disease at 27 years old. Her story, among others, inspired the creation of our Early Diagnosis and

Detection Committee with Ben Mitchell as the chair. This committee hopes to end the diagnostic odyssey for other families waiting for answers.

As we we plan for 2023, we are more motivated than ever with the right tools and people in place to make progress. And, we continue to stay devoted because of you.



Together, we dream that one day, we will prevent, treat, and cure TANGO2 disease. Until then, we remain relentless in our endeavor to accelerate TANGO2 research and support TANGO2 families. Thank for your energy, heart and commitment to this collective dream.

Ann Geffen

HANNA



WWW.TANGO2RESEARCH.ORG