

T2RF Research Grant Cycle 5

Requests for Applications Funding Year - July 1, 2025 DATE OPEN: September 2, 2024 | FULL APPLICATION DEADLINE: February 28, 2024

I. Overview

The TANGO2 Research Foundation (T2RF) supports researchers and scientists at various stages of their careers in order to advance the understanding of TANGO2 deficiency disorder (TDD) and its basic disease mechanisms, identify innovative approaches to therapeutics, and develop strategies that support patients living with TDD. The T2RF Grant Cycle 5 awards provide essential funding for seed or pilot research projects, fostering the exploration of groundbreaking research concepts. These awards support initial testing phases, allowing researchers to embark on smaller-scale endeavors. The emphasis lies in evaluating feasibility and refining methodologies, shaping the trajectory of potential larger-scale studies. Through the T2RF Grant Cycle 5, we empower researchers to take innovative ideas from conception to a vital stage of validation, contributing to the advancement of knowledge and discovery. We seek to fund rigorous, high-quality research studies in efforts to fulfill our foundation's mission enthusiastically.

A. Important Dates

The proposed schedule of events subject to the RFP is outlined below:

Public notice Deadline for questions Deadline for letter of intent (LOI) Notification to submit full proposal Deadline for full proposals Estimated Notice of Award Project Start Date September 2, 2024 September 30, 2024 November 8, 2024 5pm (EST) December 6, 2024 February 28, 2025 5pm (EST) May/June 2025 July 1, 2025

II. Introduction

For half a decade, The T2RF has been successful in carrying out its mission to fund innovative basic and clinical research conducted by talented investigators worldwide. The T2RF has awarded close to \$860,000 since its inception, funding projects that have the potential to advance the field and have an impact on children and young adults living with TDD as rapidly as possible. The T2RF research grant cycle 5 is our ongoing commitment to the cultivation of resources that accelerate scientific advancements for TDD while facilitating the next generation of TDD rare disease researchers. The T2RF research grant funds research that is innovative, collaborative and translational to positively impact treatments and potential cures for those affected by TDD. Our funding provides a platform to celebrate these scientists, spread awareness of their efforts, and highlight the importance of their research and its impact on the TDD community.

The T2RF reminds prospective applicants that the priority areas of interest exist to encourage submissions on a topic, not to limit submissions to the specified priority topics. The T2RF welcomes proposals on <u>all</u> topics related to TDD that fall within the guidelines of the grant program. This funding cycle the foundation has identified these priority topics for proposals:

- A. Understanding cellular response to therapeutics (e.g. drug, nutrition, and gene therapies)
- B. Optimizing nutrition in those affected by TDD (e.g. optimization of B vitamins)
- C. Understanding, managing and avoiding the life-threatening symptoms of TDD (e.g. metabolic crises, rhabdomyolysis)
- D. Improving affected patients' function, quality of life, and independence (e.g. language, behavior, movement and neurological symptoms of TDD)
- E. Development and Validation of Process Outcome Measures

Suitable research topics may focus on the following. *Examples include but are not limited to:*

- A. Cellular and molecular mechanisms disrupted in TDD
- B. Development and characterization of novel animal (vertebrate or invertebrate), cellular, and computational models
- C. Biomarker discovery or development
- D. Therapeutic target identification and validation (e.g. B5, other vitamins quantity/rate)
- E. Bioinformatics, machine learning, and other computational biology approaches applied to existing TANGO2 clinical, imaging, or -omics datasets
- F. Development of novel diagnostics or clinical outcome measures, including digital tools for passive or active data collection.

The T2RF strongly advocates for patient-centered research. The LOIs and full proposals submitted will be reviewed by both professional and parent/family member TANGO2 stakeholders.

III. Grant Specifics

A. Eligibility

Applicants must meet the following criteria to be eligible for this grant funding:

- A clinical, basic, or community researcher with a terminal degree (MD, DO, Ph.D., NP, or international equivalent) working in an academic medical institution or nonprofit/research institution.
- At least 3 years experience of initiating and leading (PD/PI or Co-I roles) rare disease research is preferred.
- Eligible applicants who will be named as the project's Principal Investigator are allowed to hold only one grant from T2RF per funding cycle. Note PD/PIs can be listed as a Co-Investigator (CO-I) on other collaborative project LOIs and proposals.
- Postdoctoral researchers are not eligible to apply unless they can provide a letter of employment verifying they will begin a faculty position at, or equivalent to, the assistant professor level by the beginning of the funding period in Fall 2025.

The T2RF is committed to increasing the diversity of the scientific workforce and encourages applications by researchers from underrepresented or historically excluded populations, those based outside of the U.S., not previously funded by the T2RF, as well as Early Stage Investigators as defined by the <u>National Institutes of Health (NIH)</u>.

B. Award

- This funding cycle T2RF will fund projects via the following mechanisms, by either a:
 - **Single-institution (single or multi-PI)** Award amount up to \$25,000 USD
 - This grant supports a research initiative within a single institution that may or may not involve multiple research investigators and/or a multi-departmental research team.
 - Multi-institutional (multi-PI) Award amount up to \$50,000 USD
 - This grant supports a collaborative research initiative conducted in conjunction with an institution(s) or with personnel *not* affiliated with the awarded PI/PD's academic/research institution.
 - Joint proposals within and outside the US are encouraged
- Indirect costs are unallowable
- 1-year grant duration (7/1/2025 6/30/2026)

- with the opportunity for a competitive one year renewal (up to an additional \$25,000 USD).
- No-cost extensions (NCE) can be requested and are subject to foundation approval.

C. Letters of Intent (LOI)

All grant applicants **must** submit an LOI. LOIs will be accepted starting **September 30, 2024** through **November 8, 2024 5pm EST.** Applicants will complete the T2RF Cycle 5 LOI fillable form via Submittable. The following information will be requested in the LOI fillable form and shall not exceed 5 pages, excluding NIH formatted Biosketches.

Applicant name, credentials, title (Note: Applicant submitting LOI must be the project's PD/PI) Applicant academic medical Institution, mailing address, email address, phone number

Please provide the following if applicable: Co-Investigator(s) Name, Credentials, title Co-Investigator(s) academic medical institution, mailing address, email address, phone number

Single or Multi-Institutional Award

Project Title

Lay summary of the project (not to exceed 7-10 sentences)

Project Overview (not to exceed 2 pages) - Briefly describe the project to be funded, providing the following information: relevance, purpose, rationale, specific research aims & objectives, concise methodology, expected outcomes, and main activities. Include evidence of your prior work as it relates to the proposal. Please specify if human subjects, live vertebrate animals, biological agents or recombinant DNA will be involved in the study and if approval for the study is in place through the Institutional Review Board, Institutional Animal Care and Use Committee, Radiation Safety Committee, and/or the Institutional Biosafety Committee.

Budget & Budget Narrative (not to exceed 2 pages) - Provide a brief and succinct budget narrative and budget. The budget narrative should breakdown costs for each of the major cost categories (ie., salaries, fringe benefits, equipment, supplies, and other direct costs). Describe each line item in the budget and show the calculations used to derive the costs. Expenses should be

justified in the context of the LOI proposal. List any other funding sources that will be applied to this project.

Funding Restrictions - T2RF Cycle 5 grant funds cannot be used for the following:

- Starting a new business
- Paying back loans
- School tuition
- Registry maintenance or development
- Fundraising
- Personal use of goods or services
- Travel costs
- Equipment (more than \$3,000 USD)
- Facilities and Administration (F&A) costs
- Visas and/or Passports
- Research Patient Care
- Conference/Symposium/Other event registration fees
- Membership dues

Compliance Statement (not to exceed 2-3 sentences) - Please provide a statement outlining the compliance practice for your proposed project highlighting if this work was approved by the appropriate governing bodies.

Research Compliance Bodies:

- The **Institutional Review Board (IRB)** functions to assure that research involving human subjects is carried out in an ethical manner. The principles of respect for persons, beneficence, and justice will prevail in IRB review of research involving human subjects.
- The **Institutional Animal Care and Use Committee (IACUC)** oversees the Animal Care and Use Program, Vertebrate Animal Housing and Facilities, and Live Vertebrate Animal Procedures.
- The **Institutional Biosafety Committee (IBC)** provides guidance to researchers who use biological agents or recombinant DNA within the scope of their work.
- The **Radiation Safety Committee** has the responsibility to oversee projects utilizing radioactive materials or radiation sources.

Conflict of Interest (not to exceed 2-3 sentences) - All Investigators are required to disclose their outside financial interests related to the proposed project at the time of LOI submission, on an annual basis in the event the project is funded and on an ad hoc basis as changes in their situation arise.

Biosketch - Please attach a Biographical Sketch/Curriculum Vitae (CV) in <u>NIH format</u> of the project's Principal Investigator(s). T2RF staff and peer reviewers utilize the biosketch to ensure that individuals included on the applications are equipped with the skills, knowledge, and resources necessary to carry out the proposed research.

The T2RF internal review committee of professionals and patient/family stakeholders will review LOIs. Full proposals will be requested based on LOIs meeting LOI submission requirements, project's potential impact, likelihood of successful project completion and other factors. Successful candidates will be notified by the T2RF Research Engagement Director via email to complete a full proposal application via Submittable.

Submit your T2RF Cycle 5 Grant Letter of Intent (LOI) here