

Questions? Contact edritz.javelosa@cmtrf.org

Program Name: 2025-2026 Young Researcher Innovation Grants (YRIG)

Grant Opportunity: YRIG Pilot Grant

Website: https://cmtrf.org/young-researchers-innovation-grant/

Brief Program Overview/Description: YRIG Pilot Grants are intended to support early-career researchers in the conduct of research projects aimed at testing new and innovative ideas that will expand and advance drug development for Charcot-Marie-Tooth disease, bringing new talent and thinking into the CMT research arena. YRIG Pilot Grants aim to spur innovative ideas in CMT research from early-career researchers in order to generate meaningful pilot data that can be used to launch larger CMT research projects. For successful projects, follow-on funding from CMTRF may be a possibility.

Funding Amount: Funding of up to \$75,000. This amount includes requests for indirect costs, which cannot exceed ten percent (10%) the total budget request. Awards are intended for a one (1) year duration.

Eligibility:

- Applicants must hold a doctoral degree (MD, PhD, etc.) and be no more than 10 years after receipt of terminal degree.
- Applicants must have the resources and ability to conduct independent research or will have their own lab by the proposed start of the award (by or before September 2025). Applicants who do not have their own lab or cannot conduct their experiments independently should identify and have a letter of support from a PI sponsor/mentor.
- Work may be conducted in academic/non-profit research institutions or industry (pharma/biotech).
- For industry applicants, academic-industry collaborations are encouraged, but not mandatory.
- Applications must focus on one or more of the research areas of interest outlined in Section V below.
- U.S. residents and applicants from outside the U.S. are welcome to apply.

Key Dates:

October 15-December 13, 2024	OPTIONAL Letter Of Intent (LOI) submissions period open*
December 9, 2024-February 28, 2025	Applications submissions period open
March 1-May 31, 2025	Applications reviewed
June 2, 2025	Award letters issued Contract negotiation & signature
July 1, 2025	Earliest project start date

^{*}Option for applicants to receive preliminary feedback on proposed research

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I. Charcot-Marie-Tooth Disease and the CMT Research Foundation (CMTRF)

CMTRF is a patient-led, non-profit organization focused on advancing the development of treatments and cures for Charcot-Marie-Tooth disease (CMT). CMT is a progressive, degenerative disease involving the peripheral nerves that branch out from the brain and spinal cord to other parts of the body, including the arms, hands, legs and feet. This results in numbness, sensory loss, muscle weakness/atrophy and nerve degeneration. Over time, the muscles weaken and deteriorate. Symptoms may begin as early as birth or during adulthood, and they become gradually worse over time. Adding to the complexity of CMT, here are multiple forms, with varying prevalence and different genetic mutations and molecular mechanisms. Collectively, CMT affects one in 2,500 people (about the same prevalence as multiple sclerosis), including 150,000 Americans and more than 3 million people around the world.

There is no effective treatment or cure for CMT. CMTRF's focus is to support projects that can lead to new therapeutics for CMT or greatly advance the speed and volume of CMT drug development. CMTRF grant opportunities have funded 25 projects so far, supporting promising CMT research in academia, industry and other entities. To date, CMTRF has invested over \$8M in research, and contributed to progress of 9 pipeline therapeutics for CMT treatment. You can learn more about CMT here.

II. Program and Award Overview

For the 2025-2026 grant cycle, CMTRF is launching the Young Researchers Innovation Grants (YRIG) program Pilot Grants (for postdoctoral/early career investigators). The core goals of this award program are:

- To bring new talent and thinking into the CMT research arena, funding research that is rooted in good science with high potential.
- To spur innovative ideas in CMT research from early-career researchers.
- To generate meaningful preliminary data that can be used to launch larger CMT research projects.

III. Funding Amounts

- Up to \$75,000 may be requested. Award are intended for a one-year duration.
- This amount includes requests for indirect costs, which cannot exceed ten percent (10%) of the total budget request.
- Salary support can account only for 50% or less of the amount requested. The majority of funds requested must be used for research expenses.
- Applicants may request up to \$1,500 for travel during the award and are highly encouraged to use this to attend a future Global CMT Research Convention. For YRIG awardees, CMTRF will be provided free registration for the 2025 and 2026 Global CMT Research Conventions.

IV. Eligibility

- Applicants must hold a doctoral degree (MD, PhD, etc.) and be no more than 10 years after receipt of terminal degree.
- Applicants must have the resources and ability to conduct independent research or will have their
 own lab by the proposed start of the award (by or before September 2025). Applicants not meeting
 this requirement will require a PI sponsor/mentor.
- Work may be conducted in academic/non-profit research institutions or industry (pharma/biotech)
 entities.
- For industry applicants, academic-industry collaborations are encouraged, but not mandatory.
- Applications must focus on one or more of the research areas of interest outlined in Section VI below
- U.S. residents and applicants from outside the U.S. are welcome to apply.

V. Goals of Research Currently of Interest to CMTRF/Priority Areas for YRIG

For the YRIG, research projects must include innovative approaches that address major challenges or gaps that exist for CMT drug development. Collaborative projects or efforts across research groups and industries are highly encouraged. All projects must:

- 1. Focus on one of the following Priority Areas:
 - Drug delivery to the PNS (understanding the blood nerve barrier (BNB) and/or cell types impacted by CMT (e.g. Schwann cells and peripheral axons) and how they can be targeted for therapeutic delivery, testing new delivery approaches not previously explored for the PNS, optimizing current drug delivery approaches to be more specific to the PNS, etc.)
 - Identifying new therapeutics or drug targets (can be specific to an individual subtype or aim to find ones that span across multiple subtypes)
 - Translating research to the clinic (generating better research models, identifying or validating new biomarkers and outcome measures, other activities to improve clinical trial readiness, etc.)
 - Other areas that can have a strong impact on CMT drug development that have been pre-approved by CMTRF following the submission of a Letter of Intent (LOI)
- 2. Be basic, translational or clinical research that is applicable to CMT drug development, which can be specific to an individual CMT subtype or more broadly-focused to benefit across many CMT forms.

VI. Project Review Process and Criteria

- 1. Applications will be initially reviewed internally by CMTRF staff to ensure they are complete, and that they meet the goals of the program. Those that do not meet these requirements may be triaged.
- 2. Applications will be evaluated by the YRIG committee, which is composed of members from CMTRF's Scientific Advisory Board and Research Advisory Committee, on the following criteria:
 - a. Significance: Does the project have the potential to advance scientific knowledge, address a critical challenge in the CMT drug development, and lead to meaningful outcomes that could impact or lead to future research, treatments, or further understanding of the subject area?
 - b. Innovation: Is the project idea, approach, or technology something that is not being explored or addressed by others in the CMT field?
 - c. Approach: How scientifically sound and feasible is the project plan?
 - d. Investigator Environment: Does the applicant have the expertise/resources necessary to carry out the project? Does the applicant have an interest in continuing or advancing the project after the award?
- 3. Projects will be nominated for an award based on the overall score from the YRIG committee. The number of awards given is dependent on availability of funds.
- 4. All awards are subject to compliance with applicable regulations and CMTRF policies and are contingent upon agreeable contract terms.

Chief causes for assigning low priority review scores to applications include the following:

- Insufficient information or documentation
- Inadequate statement of hypothesis, experimental design or methods
- Failure to demonstrate awareness of and plans for coping problems and pitfalls
- Insufficient or improper controls
- Failure to describe potential relevance of the proposed study to issues in CMT
- Failure to document the necessary skills or training to accomplish the goals of the proposal
- Failure of the applicant to meet all of the criteria described in this RFA

VII. Letter of Intent Submission Process - OPTIONAL

Submission period open: October 15 - December 13, 2024.

Prior to submitting a full application, applicants are highly encouraged to submit an OPTIONAL short 2-3 page Letter of Intent (LOI) for the idea of their project ahead of submitting their full proposal so that CMTRF staff and YRIG committee can review the idea and provide initial feedback and suggestions that can be incorporated into the full application.

LOIs can be submitted at any time starting on October 15, 2024.

Feedback will be provided within 4 weeks. Excludes holidays.

If you choose to skip the LOI stage, this will not negatively impact the review process for your full application.

If you choose to submit an LOI, do so at: https://webportalapp.com/webform/ryig-loi
No registration is required for the LOI submission site.

The LOI asks for the following:

- Your information
- PI sponsor/mentor information (if applicable)
- Project areas of focus
- Project title
- Expected proposed project budget
- Two to three-page project summary including a one paragraph summary; rationale; aims; preliminary results; research plan; timeframe; references.

VIII. Application Submission Process

Submission period open: December 9, 2024 - February 28, 2025, by 5:00 PM (Eastern)

A link to the online YRIG Submission Portal will be shared on December 9, 2024.

- You will be asked to create a username and password
- You will be asked to complete a profile prior to submitting an application.
- Once logged in, you will land on your personal YRIG applications portal home page. This provides a full description of what to prepare for your application.
- Once you start an application you can save it as "Draft", log out, and return later to complete it.
- Documents should be typed using:
 - Font: Times New Roman 12 or Arial 11
 - Margins: No less than a half inch on each side

Once you are logged in as described above, the following instructions walk you through the individual sections of the application.

APPLICATION TYPE

Check "Pilot Grant".

APPLICANT INFORMATION

Complete your personal details as requested.

PI SPONSOR/MENTOR INFORMATION (if applicable)

If you do not have an independent laboratory for the conduct of the proposed research, provide the requested information for the individual who will serve as your mentor or sponsor for the conduct of the research in the grant.

If you already have an independent laboratory, you are not required to have a mentor; but you have the option to identify a senior scientist who provides collaborative mentorship for you.

PROJECT DETAILS

- 1. Title of Project.
- 2. Lay Summary (one paragraph): This should be a brief overview of the project that can be understood by a non-scientist. This statement may be used to inform the non-scientific departments of CMTRF and the general public of the nature of this work. Applicants should not include any confidential or proprietary information, including intellectual property, in the lay abstract.
- 3. Scientific Summary (one paragraph): This should be a brief overview of the project and can be in scientific language. This statement will be used to inform the scientific community.
- 4. Research Focus Areas: select those that most closely describe your proposed study.
 - a. Primary Area of Scientific Focus: check one
 - b. Research Stage (bench-to-bedside): check one

APPLICATION DOCUMENTS

Items marked *** below can be prepared separately and then should be uploaded to the application as files. For file uploads, most electronic formats are accepted (PDF, Word, Excel, png...)

- Information should be presented in a clear, specific and concise manner.
- Key figures and legends must be included in the Scientific Proposal and should be of sufficient quality and size to be evaluated by the reviewer.

1. Scientific Proposal: Limit 5 pages***

This must include

- a. Aims and Hypothesis 1 page limit.
- b. Background and Research Plan 4-page limit.
- c. References, up to 2 pages.
- **a. Aims and Hypothesis:** State concisely and realistically the intent of the proposed research, structured as Specific Aims, and define the hypothesis to be tested.
- b. Background and Research Plan: This should include the following.
 - Background: Critically evaluate existing knowledge and specifically identify the gaps the project is
 intended to fill. Concisely state the importance and rationale of this research by relating the specific
 aims to longer-term objectives. This section should also show the potential importance of the
 proposed work to CMT, in particular those areas listed as areas of special interest in this RFA. In
 addition, describe the relationship of the proposed work to your long-term career goals as they apply
 to CMT.
 - <u>Preliminary Results</u>: If applicable, provide any preliminary results.
 - Experimental Design and Methods: Provide details of experimental design and methods to be used to accomplish the specific aims. Describe protocols and methods; if these are novel, describe why they might be better than current approaches. Discuss the data expected to be obtained and the means by which data will be analyzed and interpreted. Discuss potential pitfalls and/or limitations of the proposed procedures and alternative approaches to achieve the aims.
 - <u>Facilities and Equipment Available</u>: Describe resources to be used for this project, such as laboratory, clinical, animal, computer, office, etc. Provide any additional information about the

environment, including any support services available that will be utilized. If facilities or equipment at a consultant's or collaborative site will be used, they should be identified and clearly described.

- Consultants: If the proposed project includes consultant arrangements and/or collaboration with other individuals outside the applicant's group, describe the working relationships and support this description via a letter from these collaborating individual(s).
- **c. References:** Literature cited should be numbered in the sequence that they appear in the text and listed at the end of the Research Plan. Provide authors, title, journal/book, page number, year, PMID number.

2. Proposed Budget***

Pilot Grant applicants may request up to \$75,000.

Budget requests must be in US dollars.

- a. Total Amount: Fill in the total amount in the box as requested.
- b. Detailed Budget with Justification***

 Use budget template provide by CMTRF link posted at https://cmtrf.org/young-researchers-innovation-grant/ fill out the template, create a PDF of this, and upload as indicated.

3. Gantt Chart***

A clear visual timeline for your milestones, and the conduct/completion of the proposed work. Use template provide by CMTRF - link posted at https://cmtrf.org/young-researchers-innovation-grant/ - fill out the template, create a PDF of this, and upload as indicated.

4. NIH Biosketches***

These must be provided for the applicant, PI Sponsor/Mentor (if applicable), and any other key collaborators who are referenced in the application. This includes international applicants.

Format must follow the NIH Biosketch link (this includes international applicants) which can be found at https://grants.nih.gov/grants-process/write-application/forms-directory/biosketch

5. Letter of Support

Applicants who do not have their own lab or cannot conduct their experiments independently must provide a letter of support from a PI sponsor/mentor. This should specify the applicant's availability of/access to sufficient resources to conduct the proposed research.

If you already have an independent laboratory, you are not required to have a mentor; but you have the option to identify a senior scientist who provides collaborative mentorship for you.

6. Institutional Review Board/IACUC Approvals***

Where applicable, CMTRF requires that U.S.-based awardees obtain Institutional Review Board (IRB) approvals in accordance with federal policies for <u>human subjects research</u> and <u>animal research</u>. Copies of these approvals, if available, must be uploaded with the application. If pending, provide expected review/approval dates. CMTRF will not release payments to awardee institutions until these documents are received and on file at CMTRF.

OTHER SUPPORT

List any other sources of research funding you have (current and pending). Include grants, stipends, scholarships, etc. Information on other support assists CMTRF in the identification and resolution of potential sources of overlap. Commitment of an individual's effort greater than 100 percent, is not permitted.

SUBMISSION

Before clicking "Submit", be sure that the application is complete. You will receive an automated email from CMTRF immediately to let you know the application has been submitted, and the status of your application will display "Submitted" on your portal landing page. If you submit your incomplete application in error, contact edritz.javelosa@cmtrf.org.

IX. Resources and Other Information

For the latest updates and news about this RFA, visit: https://cmtrf.org/young-researchers-innovation-grant/

X. Contact Information

For technical support and grant information: Edritz Javelosa, PhD, Scientific Research Manager, CMTRF edritz.javelosa@cmtrf.org

For scientific and project applicability questions: Riann Egusquiza, PhD, Director of Research, CMTRF riann.egusquiza@cmtrf.org