



CMT RESEARCH FOUNDATION

FUNDING FACT SHEET

What does the CMT Research Foundation fund?

“We fund projects with the highest likelihood of leading to clinical trials and approved treatments in the near term. All investments are gated, meaning projects need to achieve milestones defined for each phase of work before receiving funding for the next. Every contract includes a return on the CMT Research Foundation’s initial investment which is then reinvested in new projects.”

They maintain several research priorities:

- Advancing gene therapy
 - Control the expression of genetic causes of CMT through gene editing, silencing, epigenetics, splice modification, and other approaches, including both gene therapy and small molecule approaches.
 - Understand and control modifier genes that impact disease severity and/or progression in CMT.
- Promoting nerve health
 - Understand and pharmacologically control cellular and molecular pathways that affect axon health, including survival, regeneration, and target connectivity.
 - Understand and pharmacologically control cellular and molecular pathways that affect peripheral myelination, including Schwann cell survival and function.
- Overcoming the delivery challenge
 - Understand and manipulate the peripheral nerve barriers
 - Develop novel delivery mechanisms.
 - Refine existing delivery methods
- De-risking clinical trials (secondary priority)
 - Increase the rigor of CMT research with an eye toward enhancing its drug development value.
 - Expand data and sample sharing in CMT research.
 - Define the health economics of CMT, including global incidence/prevalence, economic burden and effect of therapeutics.
 - Develop existing or novel outcome measures and biomarkers that reliably measure and predict disease progression in order to support and accelerate clinical trials.

Mission of the CMT Research Foundation

Mission: “The CMT Research Foundation is a patient-led, non-profit with one single mission: to raise funds to invest in science that will lead to treatments and cures for CMT.

We strive to address the urgent needs of the 150,000 Americans and over 3 million individuals worldwide affected by this condition—which impacts one in 2,500 people, comparable to the prevalence of multiple sclerosis.”

More about the CMT Research Foundation

CMT Research Foundation homepage: <https://cmtrf.org>

What is Charcot-Marie-Tooth Disease?: <https://cmtrf.org/what-is-cmt-disease/>

Drug Development Process and Therapy Pipeline: <https://cmtrf.org/research/cmt-research-pipeline/>

CMT news: <https://cmtrf.org/news/>

CMT stories: <https://cmtrf.org/what-is-cmt/cmt-stories/>

Application information

Full proposals are accepted on a quarterly basis

- Q1: January 27
- Q2: March 24
- Q3: June 23
- Q4: August 25

Before applying, you must submit a Letter of Intent.

- A 1-2 page letter
- Highlights the specific aims of the project and milestones expected to be reached at each stage
- Identify any collaborations that may advance findings or co-fund the project
- Evaluated against the foundation’s missions and objectives

Full proposals must include:

- Lay summary (introduction prompts/explanation in laymen terms)
- Scientific Summary and Rationale (significance prompts and research plan prompts can help)
- Specific aims (research prompts)
- Background
- Preliminary results
- Detailed experimental plan
- References

- Gantt Chart of proposed experimental plan
 - a chart in which a series of horizontal lines shows the amount of work done or production completed in certain periods of time in relation to the amount planned for those periods
- Detailed budget with justification (will fund indirect costs up to 10%)
- NIH biosketch for the research teams
- Current and pending other support

Other opportunities

Young Researchers Innovation Grant: This program aims to provide funding for small, high-impact projects that address key barriers in CMT drug development. The program is designed to support promising young scientist to conduct CMT research and produce meaningful preliminary data that can be used to kickstart larger CMT projects that aim to optimize, advance or support CMT drug development. Collaborative projects between research groups and between academic and industry investigators are highly encouraged.

The program offers two types of grants:

- Predoctoral (open to current PhD and MD students)
- Pilot Grants (open to postdoctoral fellows and early-career investigators in both academia and industry)

Research projects that aim to be funded should focus on innovative ideas and approaches:

- Understanding the blood-nerve barrier and cell types impacted by CMT as a means to optimize drug delivery in the peripheral nervous system.
- Testing new drug delivery technologies and methods for PNS delivery and activity.
- Identifying or validating new therapeutics or targets for CMT for individual subtypes or across multiple types.
- Developing improved disease models or conducting other activities to grow or speed up CMT drug development and/or improve translation of therapeutics to human clinical trials.

<https://cmtrf.org/research/young-researchers-innovation-grant/>

Where to find previously funded and active projects from CMTRF

CMTRF keeps a repository of their active projects, as well as ones that have been completed. You can find them in their website's "Research We Fund" tab: <https://cmtrf.org/research-we-fund/>