

Call for research projects 2025

"Charcot-Marie-Tooth disease"

Biomedical Sciences: therapeutic approaches

Social Sciences and Humanities: quality of life improvement

Maximum budget: **25 000 €** - Maximum duration: **24 months**

Deadline: **May 22^d, 2025 - 5pm** (Paris time)

Your profile must be created on the application platform prior to any application (instructions p3).

Context

Due to the specificities of the field of rare diseases, the CMT-France Association and the Fondation Maladies Rares (Foundation For Rare Diseases - FFRD) have agreed on a partnership to **support** and **stimulate research** on **Charcot-Marie-Tooth** disease (CMT) (rare diseases group: rare neurological diseases; rare disease network: BRAIN-Team, Filnemus, Euro-NMD).

CMT France ¹, Created in 1990 by volunteer patients, CMT-France is a non-profit association, recognized as being of general interest. With its 18 regional delegates, CMT-France is present throughout the country, as close as possible to its members.

With more than 2,000 members and supporters, CMT-France represents approximately 40,000 people affected by Charcot-Marie-Tooth disease and similar neuropathies in France.

Our team, made up of volunteer patients and parents of patients, has the following main missions:

- To be an actor in scientific and medical research.
- To help and support patients and their loved ones.
- To inform, raise awareness, and make the disease known.

FFRD² is a private non-profit organization, founded in 2012³ by five members⁴, with the aim of helping to (i) deciphering rare diseases to facilitate diagnosis and accelerate the development of new treatments, (ii) improve the daily lives of people with disease and their loved ones. Its mission is of general interest: to lead, coordinate and support research on rare diseases. It provides free support to patient organizations in their efforts to promote rare diseases research, for example through joint calls for projects such as the one proposed here.

Aim of the call

Charcot-Marie-Tooth diseases (CMT) are genetic, hereditary, neuromuscular, progressive diseases which do not affect life expectancy. They affect the peripheral nerves, essentially causing amyotrophy of the calves, forearms and hands.

The age at which symptoms appear varies widely, most often in childhood or young adulthood. Deformities and amyotrophy are variable. On the other hand, certain symptoms are common: fatigue, unstable balance, difficulty standing, difficulty climbing stairs and reduced manual dexterity. Cramps are common, especially during the course of the disease.

¹ <https://cmt-france.org/>

² <https://fondation-maladiesrares.org/en/>

³ PNMR2 : <http://www.sante.gouv.fr/le-plan-national-maladies-rares-2011-14-une-ambition-renouvelee.html>

⁴ AFM-Telethon, Rare Diseases Alliance, Inserm, Conference of University Hospital Directors, Conference of University Presidents

There are different forms of CMT, depending on the nature of the peripheral nerve damage (axonal form, demyelinating form and intermediate form). To date, more than 130 genes have been identified as being responsible for CMT.

The large number of genes involved in CMT, and their clinical heterogeneity make the search for therapeutic approaches complex. Multidisciplinary care is based on physical rehabilitation, physiotherapy, orthopaedic devices and family, psychological and social care.

This call aims to support **innovative, fundamental, translational or clinical research programs** on CMT. All disciplines in Biomedical Sciences and Social Sciences and Humanities are eligible.

The association wishes to support one project, at least, in each of the following category:

- **Development of therapeutic strategies,**
- **Improvement of everyday life quality.**

Eligibility criteria

The project must explicitly formulate a **research question**, addressing issues specifically related to CMT.

The project must demonstrate its **originality**, feasibility and **the expertise(s) of the** researchers involved.

The principal investigator of the project ("lead applicant" – P.I.) must be a **researcher**, belonging to a French or foreign public research organization⁵.

It is expected that the bearer will be statutory and hold a permanent position. Otherwise, the applicant will have to provide proof of an employment contract with the organization managing the allocated funds. The said employment contract must run **for the entire duration of the research project plus 6 months**.

There must be a clear match between the main methodology used in the project and the expertise of the principal investigator.

Financing

This call for research projects provides for financial support up to **25 000 €**, for a maximum duration of **24 months**.

Funding support can cover, **if it is entirely and exclusively dedicated to the research experiments**, the costs of:

- Fixed-term contract staff (excluding administrative staff).
- Equipment, up to a maximum of €2,000 (any excess to be justified precisely in the application form),
- Consumables,
- Travels,
- Provision of services/Subcontracting, if they relate only to the execution of a very limited part of the project (maximum 10% of the budget).

No environmental costs or management fees are eligible.

Funding must be **realistic, reasonable, detailed line by line**, and **fully justified**.

⁵ **Public research organization:** entity such as a university or research institute, or a structure dedicated to research within a health institution and associated with a university or research institute, whose primary purpose is to carry out basic research or applied research or experimental development activities and to disseminate their results through teaching, publication, or technology transfer.

An agreement will be established between the organization managing the research programme (organization of the main leader) and FFRD. **The management organization will not be able to charge any management fee on the amount granted.**

Co-financing of the selected project is possible as long as no conflict of interest exists.

The lead applicant will be responsible for the management of the allocated funds, including, where applicable, the agreement and distribution to the partner teams.

Submission Process, Selection and Scheduled

The application form, in English, must be completed at <https://ffrd.syntosolution.com/> before **May 22^d, 2025, 5pm** (Paris time).

You must have already created your profile on the application platform before submitting (a delay may be necessary). Technical support for the platform is available from 9am to 6pm until May 21st.

The joint selection by CMT France association and FFRD will take place on the basis of:

- validation of the eligibility criteria by FFRD,
- of the priorities of CMT France association,
- evaluations by independent external experts (see Annex),
- of the final scientific opinion, issued by the scientific committee (SC) of CMT France association.

The members of the board of the CMT France Association and the external experts sign a confidentiality and a non-conflict-of-interest agreement.

The reports, documents and filings sent to the members of the board and of the SC of the CMT France Association are confidential.

Scientific and financial follow-up

The CMT France Association is committed to this approach with gravity and a strong sense of responsibility. The budget that its Board of Directors will commit to the selected projects comes exclusively from donations. These include donations from members, as well as funds donated by friends and families, sometimes following the death of one of their own, in a gesture of solidarity with those who are waiting and hoping. PI and their funding management organizations are expected to adhere to this commitment.

Half of the grant will be paid at the start of the project.

A complete scientific and financial report must be produced by the PI at mid-term, in order to justify the correct progress of the work and trigger the payment of 40% of the grant.

A final scientific and financial report must be produced by the PI 1 month after the end of the project in order to trigger the payment of the remaining 10% of the grant.

Each report must be accompanied by a report in French, understandable by all members of the association, regardless of their scientific knowledge.

It is expected that the work carried out and the results obtained at the end of the project will be promoted, in the form of publications, and scientific and mainstream communications. **The project leader will make himself available for the presentation of his results at a meeting organized by the CMT France association.**

Annex

Evaluation criteria

1. Interest and importance of the research project to meet the missions of the association.

Does this study address an important problem? Innovative potential of the expected results for exploitation and for future relevant applications? Is there a sufficient body of evidence for high quality research that supports the rationale? How is the project positioned regarding international competition in the field?

2. Quality and feasibility

Soundness of the concept, clarity, originality, and pertinence of the objectives. Are the conceptual framework, design, methods techniques, and analyses adequately developed, and appropriate for the aims of the projects?

Coherence and effectiveness of the work plan, including appropriateness of the timeframe, allocation of task and resources (financial, human, and technological resources linked to the laboratory's infrastructure).

Does the applicant acknowledge potential problem areas and consider alternative strategies?

Effectiveness of the proposed measures to exploit and disseminate the project results.

3. Quality of the applicant

Ability of the applicant to conduct the research based on the quality of the CV, on its publications and on the quality of the laboratory. Competence and experience of participating research partners. Does the applicant have the right network of collaborators to undertake this type of project? If not, is it important for the Association to attract this researcher because of their skills or previous work on related subjects?

4. Budget

Budget and cost-effectiveness of the project (rational distribution of resources in relation to project's activities, partner responsibilities, and time frame).