

Call for research projects 2025

## **"Research on Friedreich ataxia "**

### *Biomedical sciences*

Maximum budget: **50 000 € to 100 000 €** - Maximum duration: **24 months**

Deadline: **June 2d, 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 rare diseases, the Fondation Maladies Rares (Foundation For Rare Diseases - FFRD) and the French Friedreich's Ataxia Association (AFAF) have agreed on a partnership to support and stimulate biomedical research on Friedreich ataxia (FA).

**AFAF**<sup>1</sup> is a patients' association, founded in 1980, run and managed by volunteers who have been closely affected by the disease. The association has around 1000 members, about half of whom are affected by the disease. Missions of AFAF are to:

- Fund research to develop treatments and improve quality of life.
- Promote information on the disease and its management to patients, caregivers, and carers.
- Support people affected by the disease in their daily lives.
- Represent patients and their families.

Since its creation, AFAF yearly funds French and international teams, conducting basic or clinical research projects (development of *in vitro* or *in vivo* models, biomarkers, improvement of daily life, pre-clinical development...). Almost 2/3 of the association budget is dedicated to support scientific research.

**FFRD**<sup>2</sup> is a private non-profit organization, founded in 2012<sup>3</sup> by five members<sup>4</sup> with the aim of helping to (i) decipher rare diseases to facilitate diagnosis and accelerate the development of new treatments, (ii) improve the daily lives of sick people and their loved ones. FFRD carries a mission of general interest: to animate, coordinate and support research on rare diseases.

### Aim of the call

Friedreich's ataxia (FA) is a mitochondrial neurodegenerative disease, whose features include general clumsiness and gait ataxia, often followed by pyramidal signs, upper-limb ataxia and dysarthria, oculomotor manifestations, nystagmus, auditory neuropathy, areflexia, distal sensory loss, dysphagia, spasticity and implies moderate to severe cardiac disorder. It also may include scoliosis and diabetes mellitus. All patients exhibit an unstable GAA expansion located in intron 1 of the FXN gene (9q21.11), encoding the protein frataxin. Although there's not yet a cure for FA, the early and multidisciplinary management is crucial to slow down its evolution and to prevent several complications.

Current pre-clinical projects are usually focused on testing new or existing molecules. Nevertheless, both researchers and clinicians agree that the effective cure of FA will reside in a "cocktail of treatments".

---

<sup>1</sup> <https://www.afaf.asso.fr/>

<sup>2</sup> [www.fondation-maladiesrares.org](http://www.fondation-maladiesrares.org)

<sup>3</sup> MRNP2: <http://www.sante.gouv.fr/le-plan-national-maladies-rares-2011-14-une-ambition-renouvelee.html>

<sup>4</sup> AFM-Telethon, Alliance Maladies Rares, Inserm, Conference Directors GChu Nationaux, Conference of University Presidents

Although a combined approach appears to be the privileged option for an effective treatment, only a few works are based on testing a combination of molecules.

AFAF is interested in supporting any research (basic, translational, clinical) in all **biomedical** disciplines. This 2025 call aims to develop **innovative research** programs to:

- better understand the pathophysiology of the disease,
- improve its diagnostic, or evaluate drug efficiency, through biomarkers,
- and/or treat FA.

*Special attention will be given to innovative\* projects which will establish **proof of concept in molecule combination to treat FA**. Those projects will aim to investigate the effects of the combination of new/approved molecules, especially if they have individually shown conclusive results on FA treatment (Omovelaxolone, Etravirine, Nicotinamide, others...).*

*\*innovative in terms of the literature and of the projects previously supported by the association (please see: <https://www.afaf.asso.fr/la-recherche/les-projets-soutenus-par-lafaf/>)*

## Eligibility criteria

The project will explicitly formulate a research **question** addressing issues specifically related to Friedreich ataxia.

The project will have to demonstrate its **novelty**, its **feasibility** and the **expertise(s)** of the researcher(s) involved.

The principal investigator ("lead applicant" – P.I.) must be a **researcher**, belonging to a French or foreign public research organization<sup>5</sup>. It is expected that **the lead applicant will have a permanent position**. Otherwise, the lead applicant will have to **provide proof of an employment contract** with the research organization managing the allocated funds. The employment contract will have to run for the entire duration of the research project, plus 6 months.

## Funding

This call for research projects provides a financial support averaging **50,000 euros and up to 100,000 euros if needed, 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,
- 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 item by item and fully justified.

An agreement will be established between the research program management organization (the lead applicant's research organization) and FFRD. **The management organization will not be able to charge any management fee on the amount awarded.**

Co-financing of the selected project is possible if there is no conflict of interest.

<sup>5</sup> **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.

**The lead applicant will be responsible for the management of the allocated funds**, including, when applicable, the agreement and distribution to the partner's team.

### Submission process, Selection and Scheduled

The application form, in English, must be completed at <https://ffrd.syntosolution.com/> before **June 2, 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 27th.*

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

- validation of the eligibility criteria by FFRD
- adequation of the proposal with patients' needs by AFAF
- evaluations by independent external expert(s) (cf. appendix)
- evaluations by AFAF/ASL-HSP/CSC joint Scientific Advisory Board

The members of the board of AFAF 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 SAB of AFAF Association are confidential.

*Please note that a rebuttal phase will take place at the beginning of September for the projects that are eligible and in line with AFAF's objectives: the evaluations of your project will be sent to you and you will have 1 to 2 weeks to submit an improved proposal.*

### Scientific and financial follow-up

*AFAF is committed to this approach with gravity and a strong sense of responsibility. The budget that its Administrative Board will commit to the selected project(s) comes exclusively from donations. These include donations from its members, but also funds donated by relatives and families, sometimes when one of their own has died, as a gesture of solidarity with those who are waiting and hoping. **The applicants are expected to adhere to this commitment.***

50% of the financial grant will be paid at the start of the project.

For projects lasting more than one year, the promoter will be required to send a brief progress report (a few lines) by e-mail every 6 months.

A full scientific and financial report must be produced by the PI halfway through the project, to justify the correct progress of the work and trigger 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 to trigger payment of the remaining 10% of the grant.

Each report must be accompanied by a plain-language summary in French that can be understood by all members of the association, whatever their scientific knowledge is.

The work carried out and the results obtained are expected to be valorized in the form of mainstream publications (at least for AFAF website and newsletter) and scientific publications (congresses and peer-reviewed journals).

Laureates will participate to AFAF annual meeting, to present their funded research.

## **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 lab 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 activities, partner's responsibilities, and time frame).