

Call for research projects "Research on Friedreich's ataxia " *Biomedical sciences*

Deadline: May 30, 2024 - 5pm (Paris time)
Budget: 50 000 € to 100 000 € - Duration: max 24 months
Contact: aap.asso@fondation-maladiesrares.com

Context

Due to the specificities of rare diseases, the Fondation Maladies Rares (Foundation For Rare Diseases - FFRD) and the French Friedreich's Ataxia Association (AFAP) have agreed on a partnership to support and stimulate biomedical research on Friedreich's ataxia (FA).

AFAP¹ is a patient's association, founded in 1980, managed by volunteers who have been closely affected by the disease. The association has more than 1000 members, about half of whom are affected by the disease. Missions of AFAP are to:

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

Since its creation, AFAP 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 quality, pre-clinical development...). Almost 2/3 of the association's budget is dedicated to support scientific research.

FFRD² is a private non-profit organization, founded in 2012³ by five members⁴ 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 situated 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 their evolution and to prevent several complications.

¹ <https://www.afaf.asso.fr/>

² www.fondation-maladiesrares.org

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

⁴ AFM-Telethon, Alliance Maladies Rares, Inserm, Conference Directors GChu Nationals, Conference of University Presidents

Current pre-clinical projects are usually focused on testing new or existing molecules. Nevertheless, both researchers and clinicians agree to state that the effective cure of FA will reside in a “cocktail of treatments”. 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 2024 call aims to **develop innovative research programs** to:

- better understand the pathophysiology of the disease,
- improve its diagnostic 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, Ninotinamide...).

Eligibility criteria

The project will explicitly formulate a research **question** addressing issues specifically related to Friedreich’s 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") must be an **academic researcher**, belonging to a research organization⁵. 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 will provide financial support averaging €50,000 per project and up to €100,000 if needed, for a maximum duration of 24 months.

Funding support can cover:

- personnel expenses – **except for permanent and/or administrative staff**,
- operations,
- missions,

provided that they are **entirely and exclusively dedicated to the research project**.

The use of service providers may only cover the execution of a very limited part of the project (max 10% of total budget), but budget overruns are possible, provided that they are justified in the project plan.

Please note that indirect costs and institutional overhead are not provided.

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.

⁵ **Research organization:** is considered a research organization, an entity such as universities or research institutes, or research structures dedicated to research within the health institution associated with a university or research institute, regardless of its legal status (public or private body) or its method of funding, 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 manage the allocated funds, including, if necessary, the agreement and allocation to the partners teams.

Submission, selection, and schedule

The proposal form (download [here](#)), in English, is to be sent by email:

- Before **May 30, 2024**, 5pm (Paris time),
- In **PDF** format (no scan),
- To aap.asso@fondation-maladiesrares.com,
- **With the following email item: "AFAF24 - Your last name"**.

The joint selection by AFAF and FFRD will be based on:

- 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.

Final decision is expected by the end of 2024.

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.***

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

For projects lasting more than one year, the PI 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.

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. Excellence

- Clarity and pertinence of the objectives,
- Credibility of the proposed approach and methodology,
- Soundness of the concept,
- Feasibility of the project,
- Competence and experience of participating research partners.

2. Impact

- Potential of the expected results for exploitation and for future relevant applications,
- Effectiveness of the proposed measures to exploit and disseminate the project results,
- Innovative potential,
- Benefit to patients, their families, and careers,

3. Quality and efficiency of the implementation

- Coherence and effectiveness of the work plan,
- Complementarity of the participants,
- Plan for sustainability of infrastructures or resources initiated by the project,
- Budget and cost-effectiveness of the project.