

Call for research projects "Research on Friedreich ataxia "

Deadline: June 22, 2023 - 5pm (Paris time)

30 000 € - Duration: 12 months

Budget 45 000 € - Duration: 18 months

60 000 € - Duration: 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 (AFAF) have agreed on a partnership to support and stimulate biomedical research on Friedreich ataxia.

AFAF¹ is a patient's association, founded in 1980, run and 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 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 models, biomarkers, improvement of daily life, treatment 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 ataxia is a neurodegenerative disorder, whose features may 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, scoliosis, spasticity, cardiac involvement, diabetes mellitus. All patients exhibit an unstable GAA expansion situated in intron 1 of the *FXN* gene (9q21.11), encoding frataxin. This disease does not

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

have a cure yet, but early and multidisciplinary management is important to slow down the evolution and avoid certain complications.

This call for projects aims to **develop innovative research program** to better understand, manage and/or treat Friedreich ataxia. All **biomedical, paramedical, and social sciences and humanities** disciplines are eligible.

AFAF is interested in supporting any research (basic, translational, clinical) that advances **understanding** of the disease, **development of therapeutic** strategies, and/or **improvement of patient's quality of life**. Due to the lack of research on those topics, special attention will be given to innovative projects which will address **incontinence or fatiguability**.

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") 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 provides for financial support of up to **60,000 euros for a maximum duration of 24 month**:

- 30 000 € - Duration: 12 months max
- 45 000 € - Duration: 18 months max
- 60 000 € - Duration: 24 months max

Funding support can cover:

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

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

The use of service providers should only cover the execution of a very limited part of the project (max 10% of total budget).

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

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

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.

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.

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 **June 22, 2023**, 5pm (Paris time)
- In **PDF** format (no scan)
- At aap.asso@fondation-maladiesrares.com
- **With the following email item:** "AFAF AAP2023 - *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 in November 2023.

Scientific and financial follow-up

A precise scientific and financial report will have to be produced **at the mid-term of the project**.

A final scientific research report, a comprehensive and readable report for patients, and a summary of the research results will have to be produced by the main applicant **1 months after the end of the project**.

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 papers.

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 carers

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