Call for research projects 2021
"MTFMT gene and Leigh syndrome"

Deadline: August 31, 2021 - 5pm (Paris time)

Budget: 10 000 € - Duration: 6-18 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 Association contre les Maladies Mitochondriales (AMMi) have agreed on a partnership to support and stimulate biomedical research on MTFMT gene and Leigh syndrome.

AMMi aims to create a network of families of patients with mitochondrial diseases, to help families by supporting them morally and materially

FFRD¹ is a private non-profit organization, founded in 2012 by five members²³ 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

The MTFMT gene (Mitochondrial methionyl-tRNA formyltransferase) is an actor in the initiation of the translation and elongation of mitochondrial proteins. Mutations in the MTFMT gene have been associated with Leigh syndrome and several pathologies related to the mitochondrial respiratory chain. Leigh syndrome or subacute necrotizing encephalomyopathy is a progressive neurological disease with a very severe prognosis. There is currently no specific treatment.

This call for projects aims to develop research on Leigh syndrome and the MTFMT gene. All biomedical disciplines are eligible.

Eligibility criteria

The project will explicitly formulate a research question addressing issues specifically related to Leigh syndrome and the MTFMT gene. The project will have to demonstrate its novelty, its feasibility and the expertise(s) of the researcher(s) involved.

¹ www.fondation-maladiesrares.org
³ AFM-Telethon, Alliance Maladies Rares, Inserm, Conference Directors GChu Nationals, Conference of University Presidents
The principal investigator ("lead applicant") must be a researcher, belonging to a research organization⁴. It is expected that the lead applicant will be statutory and in 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 duration of the research project.

**Funding**

This call for research projects provides for financial support of up to 10,000 euros for a maximum duration of 18 month.

Funding support can cover personnel expenses (excluding administrative staff), equipment, operations, and missions, provided they are entirely and exclusively dedicated to the project. The use of service providers for the project should only cover the execution of a very limited part of the project.

Funding must be realistic, reasonable, detailed post by post 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 a 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, in English, is to be sent by email:

- Before August 31, 2021, 5pm (Paris time)
- At aap.asso@fondation-maladiesrares.com
- With the following email item: "AMMi AAP 2021 - Your last name"

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

1 – validation of the eligibility criteria by FFRD
2 – adequation of the proposal with patient’s needs by AMMi
3 – evaluations by two independent external experts (cf. appendix)

**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 6 months after the end of the project.

The work carried out and the results obtained at the end of the project, in the form of publications and/or scientific papers, are expected to be enhanced.

Winners may be invited to present their results at a dedicated scientific day.

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⁴ 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.
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