

## Development of experimental models for rare diseases

Submission deadline for applications: January 9, 2024, 5:00 pm (CET)

### CONTEXT AND OBJECTIVES

The use of **non-human animal models** in scientific research and medicine is a longstanding and required practice to understand and improve human health. In the case of rare diseases as in more common pathologies, the generation of cell or animal models is a key step to characterize the biomolecular bases and pathophysiological processes of the disease.

Choosing an appropriate and reliable experimental model is of particular importance to obtain relevant results that mimic the human pathology. In some cases, it can be advantageous to use several complementary models to cover different aspects of the disease. In addition, the use of experimental models is a prerequisite to validate therapeutic proofs of concept in preclinical studies and offer new potential treatments for patients.

Mouse models are the most widely used models worldwide but, in some cases, other models offer better opportunities such as the rat model for some clinical characterization approaches or zebrafish for drug screening.

Recent technological developments offer the possibility to generate specific **induced pluripotent stem cells (iPSCs)** from patient cells providing new effective approaches for disease modelling and contributing to the respect of the 3R rule which aims at reducing the number of used animals and at replacing them when possible.

The “Fondation Maladies Rares” (Foundation For Rare Diseases – FFRD) supports the French scientific research community by launching the present call to promote the **development of new experimental models of rare diseases**, in order to:

- improve the understanding of molecular and cellular bases of rare diseases,
- provide evidence for therapeutic proofs of concept that may lead to future therapies.

## PROGRAM DESCRIPTION

This call will provide financial support to create of new experimental models for rare diseases but other requests such as breeding, phenotyping, advanced imaging, etc. are not in the scope of this call.

### Prerequisites:

- The project must rely on validated preliminary data, in particular functional studies should have been performed to demonstrate the pathogenicity of the mutation that will be reproduced on the model,
- The choice of the experimental model must be clearly justified,
- The model should be developed by a technological platform partner of the FFRD,
- For iPSCs generation, only projects for which agreements of lineage derivation have already been obtained from patients will be considered and applicants must demonstrate expertise in culture / differentiation of iPSCs.

### **This program is open to research projects covering all rare diseases.**

For rare cancers, the French National Cancer Institute (INCa) and the FFRD have defined jointly the following criteria:

- Projects concerning primary malignant tumors should be addressed to INCa,
- Projects concerning benign tumors as well as systemic rare diseases involving tumor development will be evaluated within this call.

Only one project per research team will be funded for the current call.

## ACCESS TO TECHNOLOGICAL PLATFORMS

FFRD has established partnerships with several technological platforms to generate new experimental models for rare diseases. These partners offer an outstanding range of expertise, skills and services and provide advice to select the most appropriate experimental model to develop resources for the scientific community.

Most of these platforms are part of [CELPEDIA](#), the French infrastructure that promotes innovative services and tools on model organisms.

Applicants must contact partner platforms as early as possible to ensure the feasibility of the project and obtain assistance in optimizing the technical design as the model will be developed by the platform. A technical validation of the project by the platforms is mandatory.

The list of the partner platforms is available on the FFRD website: <https://fondation-maladiesrares.org/en/plateformes-partenariats/>.

If specific needs are not covered by partner platforms, please contact the FFRD at [aap-bio@fondation-maladiesrares.com](mailto:aap-bio@fondation-maladiesrares.com) in order to evaluate the eligibility of the proposed model and conditions of services.

## ELIGIBILITY

The principal investigator of the study must belong to a French research team, affiliated to academia (research team working in universities, other higher education institutions or research institutes) and/or to clinical/public health sector (research team working in hospitals/public health organizations).

## EVALUATION

Applications will be reviewed by at least two national or international academic experts in the field and selected by a dedicated scientific committee composed of FFRD Scientific Advisory Board members and experts in the field based on the following criteria:

- Relevance and significance of the project,
- Project quality and scientific soundness,
- Feasibility of the project,
- Innovation,
- Quality of the applicant and quality of the laboratory.

## FUNDING

Funding will only cover costs of the platform (services and consumables) based on the quote provided in the application. Funding is not intended to cover equipment, operating or personnel costs from the laboratory of the applicant.

**Overheads are not allowed by the FFRD.**

## SUBMISSION AND SCHEDULE

Applications can only be submitted on the FFRD Synto online platform:  
<https://ffrd.syntosolution.com/>

Provisional schedule:

Launch of the call	November 8, 2023
Submission deadline for application	January 9, 2024 - 5:00 pm (CET)
Technical validation by platforms	January 26, 2024 - 5:00 pm (CET)
Notification of the results	End of April – beginning of May 2024

Results will be communicated by e-mail to the principal investigator.

Applicants resubmitting projects must provide a detailed answer to the comments provided by the scientific committee of the FFRD at the previous session and highlight changes in the revised version.

Applicants belonging to a research team already funded by the FFRD since 2017 must provide a detailed report on the results and impacts of all ended project(s). For ongoing projects, a detailed progress and / or preliminary data report is required.

Report forms are available on the applicant portal or upon request by e-mail at [aap-bio@fondation-maladiesrares.com](mailto:aap-bio@fondation-maladiesrares.com). Please attach all reports to the proposal in the appropriate section.

## FAIR policy / IRDiRC policies and guidelines

By submitting a project to this call, applicants will adhere to the [FAIR guiding principles for scientific data management and stewardship](#).

The aim of the call is in compliance with the goals set by the International Rare Diseases Research Consortium (IRDiRC). Applicants are expected to follow [IRDiRC policies and guidelines](#).

## COMMUNICATION

Applicants must agree that title and non-confidential abstract of funded projects as well as principal investigator name and affiliation(s) will be published on the FFRD website: <http://fondation-maladiesrares.org>.

## ACKNOWLEDGEMENT POLICY

Applicants must acknowledge the FFRD in all communications related with the project (posters, oral communication, scientific publications, etc.) as a funding source using the following terms “Foundation For Rare Diseases” or “Fondation Maladies Rares” and/or using the appropriate logo (available upon request).

Reference(s) of the publication(s) must be sent to the FFRD by e-mail to [aap-bio@fondation-maladiesrares.com](mailto:aap-bio@fondation-maladiesrares.com).

## CONTACT

Please contact [aap-bio@fondation-maladiesrares.com](mailto:aap-bio@fondation-maladiesrares.com) for any question related with this call.