FONDATION Maladies Rares

CALL FOR PROPOSALS 2025

- MODELS -

Development of experimental models for rare diseases

Proposal submission deadline: **8th January 2026, 5:00 pm (CET)**

CONTEXT AND OBJECTIVES

The use of non-human models in scientific and medical research has historically been vital to enhance our understanding of human health. The development of cellular or animal models is a critical step in identifying the biomolecular and pathophysiological mechanisms underlying rare diseases and more common conditions.

It is vital to select an appropriate and reliable experimental model to ensure that the results obtained are meaningful and closely reflect the human condition. In certain instances, the utilisation of several complementary models can prove advantageous in exploring multiple faspects of the disease. Furthermore, experimental models are essential for validating proof-of-concept studies in preclinical research, paving the way for potential new treatments.

While mouse models are the most widely used globally, other models can offer distinct advantages. For instance, rats are useful for certain clinical characterisations, while zebrafish are ideal for high-throughput drug screening.
Recent technological advances have enabled the creation of patient-derived induced pluripotent stem cells (iPSCs), providing innovative and efficient approaches to disease modelling. These advances also support the 3Rs principle (Reduce, Refine, Replace) by helping to minimise animal use and replace it wherever possible.

In this context, the Fondation Maladies Rares (FFRD) is supporting the French scientific research community by launching this call for proposals. The objective is to encourage the creation of new experimental models of rare diseases, thereby enhancing our understanding of their molecular and cellular mechanisms and providing proof-of-concept evidence that could result in future therapeutic developments.

PROGRAM DESCRIPTION

This call will provide financial support for the **creation of new experimental models** for rare diseases. Please note that all other requests, including those relating to breeding, phenotyping and advanced imaging, are outside the scope of this call.

Prerequisites:

- The project MUST rely on **validated preliminary data**. In particular, it is essential that functional studies have been performed to demonstrate the pathogenicity of the genetic variant that will be reproduced on the model,

- It is essential that the **choice of experimental model** is clearly justified.
- The model should be **developed by a technological platform partner of the FFRD** (please refer to the dedicated section for more information).

For information on iPSC projects, please refer to the dedicated section.

This programme 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 tumours should be addressed to INCa.
- Projects concerning benign tumours, as well as systemic rare diseases involving tumour development, will be evaluated within this call.

INVOLVEMENT OF TECHNOLOGICAL PLATFORMS

For this call, FFRD has established partnerships with several technology platforms. **Applicants are kindly requested to liaise with the partner platforms at the earliest opportunity** in order to confirm project feasibility and request assistance with optimising the technical design, given that the model will be developed by the platform.
Furthermore, technology platforms will be required to validate applications on the online submission platform in addition to providing a quote.

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

Working with non-partnering platforms is only permitted if the specific needs are not covered by partner platforms. If this is the case, please contact the FFRD at aap-bio@fondation-maladiesrares.com before submitting of the project.

PROJECTS AIMING AT DEVELOPING iPSC MODELS

All applicants are required to contact one of the two iPSC partner platforms as soon as the call is launched.

The following projects may be covered by the funding:

- The process of reprogramming patient cells into iPSCs,
- The creation of new models of rare diseases achieved by genome editing (knockout, knock-in or point mutation) of characterised iPSC lines*,
- The creation of isogenic controls achieved by genome editing (knockout, knock-in or point mutation).

* In order to facilitate the creation of new models of rare diseases through genome editing, applicants are strongly encouraged to use fully characterised human embryonic stem cells (hESCs) made available by partnering platforms.

If this is not possible for any reason, the applicant will be asked to provide the platform with quality control information about the iPSC to be edited well in advance of submission. If this information is provided too late, the platform may refuse the project.

The quality control information that the platform will ask for can include, but is not limited to, the following:

- Explanatory diagram showing how the master bank was generated (media, matrix, types of passages and freezing method),
- SNP analysis using Illumina chips (the platform will reanalyse the raw data),
- Broad-spectrum mycoplasma test (Eurofins PCR type),
- Genotyping of the region of interest using Sanger sequencing or NGS,
- Proof that these lines have demonstrated pluripotency.

In the event that the project involves the creation of organoids, applicants are required to demonstrate their technical and financial capacity to do so, as well as to explain how organoids offer added value in comparison to 2D models.

Please note that there are additional special requirements for iPSC projects:

- The Committee for the Protection of Persons (Comité de Protection des Personnes/CPP) must approve the project prior to submission, and the approval document must be uploaded at the time of submission.
- If applicable, the cells to be modified must be sent to the platform within three months of the successful applicants being notified, and confirmation of receipt will be obtained from the platform. Should the cells not be dispatched within the specified timeframe, the project will not be funded, and the next project on the waiting list will be funded instead.

ELIGIBILITY

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

If the project has been entirely or partially prepared using artificial intelligence, this must be clearly stated in the proposal.

FUNDING

Please note that **funding will only cover the costs of the platform** (including services and consumables), as set out in the quote provided in the proposal. Therefore, all other expenses are not eligible.
Applicants are required to include **a quote from the partnering platform** when applying.

Please note that overhead costs are not eligible for FFRD funding. Furthermore, FFRD grants are exempt from VAT.
For the current call, only one project per research team will be funded.

SUBMISSION AND SCHEDULE

Proposals must be submitted exclusively through the FFRD Synto online platform: https://ffrd.syntosolution.com/. Proposals sent by e-mail or by any other means will not be considered. The application form and the documents to be provided are detailed on the online portal.

Prior to submitting their project, candidates and their directors are required to create and complete their profiles on the Synto platform. For guidance, please refer to the tutorials available in the 'Documentation' section of the Synto platform.

Please note that technical support is only available from the moment the call is initiated until 24 hours before the submission deadline. Candidates are responsible for identifying any potential issues in good time, as no extensions will be granted after the call for proposals has closed.

All communications will henceforth be sent via email through the FFRD Synto online platform. Please ensure that emails from 'FFRD by Synto' are received in a timely manner.
Candidates are advised to regularly check their Synto account to monitor the progress of their application.

Provisional schedule:

Launch of the call	13th November 2025
Proposal submission deadline	8th January 2026 - 5:00 pm (CET)
Notification of funding decision	May 2026

Please note that projects should start within nine months of the funding decision being announced.

Applicants resubmitting projects must provide a detailed response to the comments from the FFRD scientific committee from the previous session and clearly highlight the changes made in the revised version.

Applicants from research teams that have received FFRD funding since 2017 must submit a detailed report for all completed projects if this report has not already been submitted to the FFRD. For ongoing projects, a progress report and/or preliminary data must be provided. Report forms are available on the applicant portal (tab ‘Documentation’) or upon request by e-mail at aap-bio@fondation-maladiesrares.com. Please ensure that all reports are attached to the proposal in the designated section.

EVALUATION

The FFRD will first assess the administrative and budgetary eligibility of the projects submitted, as well as their suitability within the scope of the call for proposals. Projects not meeting these criteria may be declared ineligible.

All eligible proposals will be evaluated by a minimum of two national or international academic experts in the relevant field (external reviewers). Funded proposals will then be selected by a dedicated scientific committee (internal experts). The evaluation and selection of funded proposals will be 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.

ADMINISTRATIVE AND FINANCIAL MONITORING

A research agreement will be signed between the managing institution of the selected applicants and the FFRD. This agreement will outline the scientific and financial monitoring requirements for the project and should be signed within six months of the funding decision being made. The implementation of this policy is subject to approval by the relevant ethical bodies, where applicable.
Successful applicants will be required to submit project monitoring reports as requested by the FFRD, using the templates provided, and to report on project progress upon request.

For iPSC projects, the research agreement will only be signed once the platform has confirmed receipt of the cells to be modified (within three months of notifying the successful applicants).

FAIR POLICY / IRDiRC POLICIES AND GUIDELINES

By submitting a project to this call, applicants agree to adhere to the FAIR guiding principles for scientific data management and stewardship.
The objectives of this call are in alignment with the goals set out by the International Rare Diseases Research Consortium (IRDiRC). Applicants are expected to adhere to the IRDiRC policies and guidelines.

COMMUNICATION

Applicants must agree that the title and non-confidential abstract of funded projects, along with the principal investigator's name and affiliation(s), will be published on the FFRD website: https://fondation-maladiesrares.org/projets-de-recherche-laureats/.

To accelerate research into rare diseases, the SAB of the FFRD has decided that all funded models will be published on the FFRD website and on specialised websites 18 months after their creation by the platforms.

ACKNOLEDGEMENT POLICY

Applicants must acknowledge the FFRD as a funding source in all project-related communications (posters, oral presentations, scientific publications, etc.) by using the terms “Foundation For Rare Diseases” or “Fondation Maladies Rares” and/or by including the appropriate logo (available upon request).
Please send the reference(s) of the publication(s) to the FFRD via e-mail to aap-bio@fondation-maladiesrares.com.

CONTACT

For any questions related to this call, please contact aap-bio@fondation-maladiesrares.com.