

Call for research projects 2024

Biomedical Sciences

" *Pathophysiology of osteogenesis imperfecta* "

Maximum budget: **150 000 €** - Maximum duration: **36 months**

Contact: aap.asso@fondation-maladiesrares.com

Deadline: 05th September 2024 - 5pm (Paris time)

We would like to draw your attention to the need to create your profile before connecting to the application platform (see instructions on page 3).

Context

Due to the specificities of the field of rare diseases, the VMOV Association and the Fondation Maladies Rares (Foundation For Rare Diseases - FFRD) have agreed on a **partnership to support and stimulate research on the pathophysiology of osteogenesis imperfecta**.

VMOV¹ has the dual aim of setting up initiatives to improve care for patients and their families and supporting all types of research, whether fundamental, translational or clinical, with a view to improving quality of life.

FFRD² is a private non-profit organization, founded in 2012³ by five members⁴, with the aim of helping to (i) deciphering rare diseases to facilitate diagnosis and accelerate the development of new treatments, (ii) improve the daily lives of people with disease and their loved ones. Its mission is of general interest: to lead, coordinate and support research on rare diseases. It provides free support to patient organizations in their efforts to promote rare diseases research, for example through joint calls for projects such as the one proposed here.

Aim of the call

Osteogenesis imperfecta is one of the most common rare bone diseases, with an estimated prevalence of close to 1 in 15,000, affecting around 5,000 patients in France. It primarily affects the skeleton, increasing the risk of fracture, resulting in bone pain, loss of mobility and disability.

The genetic abnormalities that cause it are numerous: **28 mutations** have currently been identified, with **highly variable penetrance**. Most often, these anomalies result in a quantitative or qualitative abnormality in type 1 collagen, but other mechanisms are also possible, although not yet fully understood. Clinically similar forms may be linked to different

¹ <https://vmov.assoconnect.com/page/820818-presentation>

² <https://fondation-maladiesrares.org/en/>

³ PNMR2 : <http://www.sante.gouv.fr/le-plan-national-maladies-rares-2011-14-une-ambition-renouvelee.html>

⁴ AFM-Telethon, Rare Diseases Alliance, Inserm, Conference of University Hospital Directors, Conference of University Presidents

genetic abnormalities, and similar genetic abnormalities may be responsible for different clinical forms. The cascade of events from the gene to its product - the production of sufficient normal collagen - is poorly understood.

This call for proposals aims to **support innovative, transdisciplinary, fundamental or translational research** programmes. **All biomedical science disciplines are eligible.**

The association wishes to support any research aimed at a **better understanding of the causes and physio-pathological mechanisms** behind Osteogenesis imperfecta, as well as the development of therapeutic strategies.

Eligibility criteria

The project must explicitly formulate a **research question**, addressing issues specifically related to the **pathophysiology of osteogenesis imperfecta**.

The project must demonstrate its **originality**, feasibility and **the expertise(s) of the researchers** involved.

The principal investigator of the project ("lead applicant" – P.I.) must be a **researcher**, belonging to a public research organization⁵. **It is expected that the P.I. will be statutory** and hold a permanent position. Otherwise, the applicant will have to provide proof of an employment contract with the organization managing the allocated funds. The said employment contract must run for the entire duration of the research project plus 6 months.

Financing

This call for research projects provides for financial support up to **150 000 €**, for a maximum duration of **36 months**.

Funding support can cover, **only if they are entirely and exclusively dedicated to the research experiments**, the costs of:

- Fixed-term contract staff (excluding administrative staff). Particular attention will be given to projects involving the funding of thesis or postdoctoral fellowship.
- Equipment,
- Consumables,
- Travels, up to a maximum of €2,000 (possible overage to be justified precisely in the response form),
- Provision of services, if they relate only to the execution of a very limited part of the project (maximum 10% of the budget).

No environmental costs or management fees are eligible.

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

⁵ **Research organisation:** is considered to be a research organisation, an entity such as a university or a research institute, or a structure dedicated to research within a health institution and associated with a university or research institute, 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 organisation managing the research programme (organisation of the main leader) and FFRD. This agreement will be drawn up and sent to the managing body, **which must sign it within 6 weeks. The management organisation will not be able to charge any management fee on the amount granted.**

Co-financing of the selected project is possible as long as no conflict of interest exists. **The lead applicant will be responsible for the management of the allocated funds**, including, where applicable, the agreement and distribution to the partner teams.

Submission Process, Selection and Scheduled

The form, in English, can be completed at <https://ffrd.syntosolution.com/>, before **5th September 2024, 5 p.m.** (Paris time).

We would like to draw your attention to the need to create your profile before connecting to the application platform (this step may take some time). Technical support on the application platform is available from 9am to 6pm (Paris time), until September 4th 2024.

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

- validation of the eligibility criteria by FFRD,
- of the priorities of the VMOV association,
- evaluations by independent external experts (see Annex),
- of the final scientific opinion issued by the scientific committee (SAB) of the VMOV association.

The members of the board and of the SAB of the VMOV Association and the external experts sign a confidentiality and a non-conflict-of-interest agreement.

The reports, documents and filings sent to the members of the board and of the SAB of the VMOV Association are confidential.

Scientific and financial follow-up

The VMOV Association is committed to this approach with gravity and a strong sense of responsibility. The budget that its Board of Directors will commit to the selected projects comes exclusively from donations. These include donations from members, as well as funds donated by friends and families, sometimes following the death of one of their own, in a gesture of solidarity with those who are waiting and hoping. PI are expected to adhere to this commitment.

30% of the grant will be paid at the start of the project.

The P.I. must send a brief e-mail at 6 months, 18 months and 30 months on the progress of the funded project (a few lines).

A full scientific and financial report must be produced by the P.I. at 12 and 24 months, in order to justify the correct progress of the work and trigger **payment of 30% of the grant for each deadline.**

A final scientific and financial report must be produced by the P.I. 1 month after the end of the project in order to trigger payment of the remaining 10% of the grant.

Each report must be accompanied by a plain-language summary in French that can be understood by all members of the association, whatever their scientific knowledge.

At the end of the project, it is expected that the work carried out and the results obtained will be promoted, in the form of publications, and scientific and general public communications. The P.I. will make himself available to present the results at a meeting organised by the VMOV association.

Annex

Evaluation criteria

1. Excellence

- Clarity and pertinence of the objectives,
- Credibility of the proposed approach and methodology,
- 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

3. Quality and efficiency of the implementation

- Coherence and effectiveness of the work plan
- Budget and cost-effectiveness of the project