

Call for research projects 2025

Merosin-deficient Congenital muscular dystrophy

Biomedical Sciences

Maximum budget: **25 000 €** - Maximum duration: **24 months**

Deadline: **May 12th 2025 - 5pm** (Paris time)

Your profile must be created on the application platform prior to any application (instructions p3).

Context

Due to the specificities of the field of rare diseases, the Association LAMA2 France and the Fondation Maladies Rares (Foundation For Rare Diseases - FFRD) have agreed on a partnership to support and stimulate research on **laminin-alpha2 -deficient congenital muscular dystrophy**.

LAMA2 France¹, is a young non-for-profit French Association founded in 2021. It aims at supporting research and research teams in France and abroad in order to develop treatments for congenital muscular dystrophy caused by laminin-alpha2 deficiency (DMC LAMA2 or MDC1A). Its activities range from raising awareness on this rare disease among the academic and medical communities, the media, the general public, individuals, public bodies and companies; working towards the creation of a registry and natural history of this dystrophy in France and in coordination with other European countries; and fostering European and international collaboration between academics, associations and private stakeholders with an interest in this rare disease. LAMA2 France already co-funded or funded several research projects, notably in 2024 as part of a first call for projects launched with FFDR. Finally, LAMA2 France co-founded LAMA2 Europe with the Dutch and Spanish 'sister' patient associations which now expanded to several European countries.

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

Laminin alpha-2–Congenital Muscular Dystrophy (LAMA2-CMD), also known as Merosin-Deficient Congenital Dystrophy Type 1A (MDC1A), is a rare congenital neuromuscular disease that affects 1 to 4 people in 100,000. It is the most common form of congenital muscular dystrophies, representing 30 to 40% of them. It is caused by mutations in the *LAMA2* gene. These mutations result in progressive muscle wasting and inflammation leading to delayed milestones, and reduced lifespan in affected patients.

While the molecular basis underlying this disease, and some pathomechanism, are known, there is currently no cure or treatment. However, since more than 10 years, several initiatives ranging from

¹ <https://www.lama2.fr/home>

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

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

establishing registries and clinical natural histories, deciphering disease pathways, developing animal models or research projects targeting possible therapeutic approaches are growing.

In this context, LAMA2 France and FFRD launch this second call for proposal that aim to support any innovative research (basic or translational) aimed at the development of therapeutic strategies. All biomedical disciplines are eligible.

This call should contribute to attract teams or researchers working on this disease, but also on other diseases where results could be transferred on LAMA2 CMD.

Eligibility criteria

The project must explicitly formulate a **research question**, addressing issues specifically related to **laminin alpha2 -deficient congenital muscular dystrophy**.

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 organization in France or abroad⁵. If the PI does not hold a permanent position, he/she will have to provide proof of 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.

There must be a clear match between the main methodology used in the project and the expertise of the principal investigator.

Financing

This call for research projects provides for financial support up to 25 000 €, **for a maximum duration of 24 months**.

Funding support can cover, **if it is entirely and exclusively dedicated to the research experiments**, the costs of:

- Fixed-term contract staff (excluding administrative staff) or gratifications.
- Equipment, up to a maximum of €2,000 (any excess to be justified precisely in the response form),
- Consumables,
- Travels, up to a maximum of €2,000,
- Publication related costs, up to a maximum of €2,000,
- 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, aligned with the usual practices of the organisation, detailed line by line, and fully justified**.

An agreement will be established between the organisation managing the research programme (PI institution) and FFRD. **The management organisation will not be able to charge any management fee on the amount granted.**

⁵ **Research organization:** is considered to be a research organization, 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.

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 application form, in English, must be completed at <https://ffrd.syntosolution.com/>, before **12th May 2025, 5 p.m.** (Paris time).

You must have already created your profile on the application platform before submitting (a delay may be necessary). Technical support for the platform is available from 9am to 6pm until 11th May 2025.

For the applicants who are re-submitting a project, they are advised to detail on what extent they took the comments of their first evaluations into account.

The joint selection by LAMA2 France and the FMR will take place on the basis of:

- The validation of the eligibility criteria by the FMR,
- the priorities defined by LAMA2 France,
- the evaluations by independent external experts (see Annex).

The members of LAMA2 France Board and the external experts sign a confidentiality and a non-conflict-of-interest agreement.

The reports, documents and files sent to the members of the board of LAMA2 France are confidential.

Scientific and financial follow-up

LAMA2 France 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 project(s) comes exclusively from donations. These include donations from members, as well as funds donated by friends and families, charity, awareness-raising events. PI are expected to adhere to this commitment.

Half of the grant will be paid at the start of the project.

For projects longer than one year, the PI will have to report briefly by email, every 6 months, on the progress of the project (a few lines).

A complete scientific and financial report must be produced by the PI at mid-term, to justify the correct progress of the work and trigger the payment of 40% of the grant.

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

Each report must be accompanied by a report in French, understandable by all members of the association, regardless of their scientific knowledge.

It is expected that the work carried out and the results obtained at the end of the project will be promoted, in the form of peer-reviewed publications, and scientific and mainstream communications. Efforts towards publication in open-access and participation at related scientific conferences will be favourably evaluated. The PI commits at least to one publication in a peer-review journal. The PI will make himself/herself available for the presentation of the project's results at a meeting organized by LAMA2 France.

Annex

Evaluation criteria

1. Interest and importance of the research project to meet the missions of the association.

Does this study address an important problem? Innovative potential of the expected results for exploitation and for future relevant applications? Is there a sufficient body of evidence for high quality research that supports the rationale? How is the project positioned regarding international competition in the field?

2. Quality and feasibility

Soundness of the concept, clarity, originality, and pertinence of the objectives. Are the conceptual framework, design, methods techniques, and analyses adequately developed, and appropriate for the aims of the projects?

Coherence and effectiveness of the work plan, including appropriateness of the timeframe, allocation of task and resources (financial, human, and technological resources linked to the laboratory's infrastructure).

Does the applicant acknowledge potential problem areas and consider alternative strategies?

Effectiveness of the proposed measures to exploit and disseminate the project results.

For applicants who re-submit to this call, integration of evaluators comments if relevant (for this, an extra page can be used for, above the limit of the application form)

3. Quality of the applicant

Ability of the applicant to conduct the research based on the quality of the CV, on its publications and on the quality of the laboratory. Competence and experience of participating research partners. Does the applicant have the right network of collaborators to undertake this type of project? If not, is it important for the Association to attract this researcher because of their skills or previous work on related subjects?

4. Budget

Budget and cost-effectiveness of the project (rational distribution of resources in relation to project's activities, partner responsibilities, and time frame).