

Call for research projects 2024

"Research on Hereditary spastic paraplegia"

Biomedical sciences

Deadline: May 30, 2024 - 5pm (Paris time)

Budget: 25 000 € - Duration: max 24 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 French Strümpell-Lorrain Hereditary Spastic Paraplegia Association (ASL-HSP) have agreed on a partnership to support and stimulate biomedical research on Hereditary Spastic Paraplegia (HSP).

ASL-HSP¹ initial aim was to bring together and help people affected by 'Hereditary Spastic Paraplegia', without excluding regions, states, or continents. The Association's mission is to welcome, unite, build solidarity, inform, advise, support, represent, raise awareness, and support research.

FFRD² is a private non-profit organization, founded in 2012³ by five members⁴ with the aim of helping to (i) decipher rare diseases to facilitate diagnosis and accelerate the development of new treatments, (ii) improve the daily life 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

Hereditary spastic paraplegia (HSP) is a genetically and clinically heterogeneous group of slowly progressive neurological disorders that can be divided into pure and complex forms⁵. The pure form is characterized by pyramidal signs predominantly affecting the lower limbs and with possible association of sphincter disturbances and deep sensory loss. The complex form is characterized by the addition of variable neurological or non-neurological features.

¹ <https://www.asl-hsp-france.org>

² www.fondation-maladiesrares.org

³ MRNP2: <http://www.sante.gouv.fr/le-plan-national-maladies-rares-2011-14-une-ambition-renouvelee.html>

⁴ AFM-Telethon, Alliance Maladies Rares, Inserm, Conference Directors GChu Nationals, Conference of University Presidents

⁵ https://www.orpha.net/consor/cgi-bin/Disease_Search.php?data_id=655&lng=en

This call for projects aims to **support any innovative research program** (basic, translational, clinical) that advances development of therapeutical strategies for HSP. All **biomedical** disciplines are eligible.

Particular attention will be given to projects in **translational and clinical research**, as well as to projects in **physiopathology interested in paraparesis due to the most common mutations** (in the *SPG-4, SPG-3A, SPG-7 and SPG-11* genes).

Eligibility criteria

The project will explicitly formulate a research **question** addressing issues specifically related to HSP.

The project will have to demonstrate its **novelty**, its **feasibility** and the **expertise(s)** of the researcher(s) involved.

The principal investigator ("lead applicant") must be an **academic researcher**, belonging to a research organization⁶. It is expected that **the lead applicant will have 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 entire duration of the research project, plus 6 months.

Funding

This call for research projects provides financial support of up to **25 000 euros for a maximum duration of 24 month**.

Funding support can cover:

- personnel expenses – **except for permanent and/or administrative staff**,
- operations,
- equipment,
- missions (limited to 2 000 €),

provided that they are **entirely and exclusively dedicated to the research project**.

The use of service providers should only cover the execution of a very limited part of the project (max 10% of total budget).

Please note that indirect costs and institutional overhead are not provided.

Funding must be realistic, reasonable, detailed item by item 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 any 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.

⁶ **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.

Submission, selection, and schedule

The proposal form, in English, is to be sent by email:

- Before **May 30, 2024**, 5pm (Paris time)
- At aap.asso@fondation-maladiesrares.com
- With the following email item: "ASL24 *Your last name*"

The joint selection by ASL-HSP and FFRD will be based on:

- validation of the eligibility criteria by FFRD
- adequation of the proposal with patients' needs by ASL-HSP
- evaluations by independent external expert(s) (cf. appendix)
- evaluations by ASL-HS/CSC/AFAF joint Scientific Advisory Board

Final decision is expected at the end of 2024.

Members of the Board of ASL-HSP and external experts sign an agreement of confidentiality and absence of conflict of interest.

All reports, documents and evaluations sent to the members of the Executive Committee and the Board of ASL-HSP Association are confidential.

Follow-up

*The ASL-HSP Association is committed to this approach with 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 its members, but also funds donated by relatives and families, sometimes when one of their loved ones has died, as a gesture of solidarity with those who are waiting and hoping. **The researchers and their management organizations are expected to adhere to this commitment.***

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 **1 month after the end of the project.**

The work carried out and the results obtained are expected to be valorised in the form of mainstream publications (at least for ASL-HSP website and newsletter) and scientific papers.

Laureates will participate to ASL-HSP annual meeting, to present their funded research.

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