

SCIENTIFIC CALL FOR PROPOSALS AFM-TELETHON 2024

THIS INTERNATIONAL CALL FOR PROPOSALS, OPEN TO BOTH FRENCH AND FOREIGN TEAMS, AIMS TO SUPPORT RESEARCH ALONG THESE THEMATICS:

A. FUNDAMENTAL RESEARCH AND PHYSIOPATHOLOGY OF DISEASES OF THE NEUROMUSCULAR SYSTEM

1 - Fundamental research projects aimed at increasing our understanding of molecular, cellular, physiological and pathological mechanisms, involving the structure and function of skeletal muscles, motor neurons, neuromuscular junctions, aging and degeneration/regeneration in physiological and pathological conditions.

For applications on cardiac research, the applicant has to justify why this is of interest for neuromuscular pathologies, for example, the use of skeletal muscle cells or a neuromuscular animal model system.

2 - Research projects aimed at increasing our understanding of the clinical and genetic heterogeneity of neuromuscular diseases.

3 - Genetic cardiomyopathies with muscle structure abnormalities.

4 - Physiopathology of smooth muscle in relation to neuromuscular diseases.

5 - Differentiation of adult, embryonic and iPS stem cells into skeletal/ cardiac muscles or neuronal cells (including motor neurons) (in physiological and pathological conditions).

6 - Genetic/epigenetic regulation of genes and characterization of gene regulation networks within the neuromuscular system.

Nota Bene: Concerning projects on ALS, priority will be given to therapeutic innovation.

B. DEVELOPMENT OF INNOVATIVE THERAPEUTIC APPROACHES FOR RARE GENETIC DISEASES

1 - Gene and/or cell therapy for rare genetic disorders

- Breakthrough innovation
- Gene transfer and gene correction
- Genome editing
- Treatments using genetically-modified cells
- Development of delivery strategies and biomaterials
- Control of the immune response (auto-immunity, anti-vector and anti-transgene responses, etc.)

2 - Strategies for modifying gene expression both at the gene level (cis, trans or epigenetic modifications) and RNA level

- Projects based on gene transfer, chemical molecules or biotherapies

3 - Pharmacotherapies of neuromuscular diseases

The proposed projects must be exclusively focused on neuromuscular diseases

4 - Translational research: tools for evaluation of treatments for neuromuscular diseases

- Outcome measures: functional, connected objects, imaging
- Genomic, transcriptomic, proteomic, metabolomic biomarkers of pathologies and/or therapies
- New cellular, tissue and animal models; organoids

PARTICULAR FOCUS on biomarkers and outcome measures to more efficiently assess neuromuscular disease drugs or medical devices.

TYPES OF FINANCING:

The selected projects will be subject to an agreement with AFM-Telethon. AFM-Telethon may decide to finance the selected project under a collaboration which entails a co-ownership of the results.

- Trampoline grant: intended to support young teams or investigators early in their professional career (less than 10 years after thesis, either permanent or non-permanent position), and/or early stage innovative and high risk projects. This grant is awarded for a maximum of 50,000 euros for one year.
- Research project for one year, renewable for a second year, and exceptionally for a third year.
- Post-doctoral fellowship for one year, renewable for a second year.
- PhD fellowship (open to students enrolled in a French university doctoral degree program) for a maximum of three years.

Please note that Fellowship salaries (PhD and post-doctoral fellowships) cannot be included in the budget of a Research grant or a Trampoline grant. PhD and post-doctoral fellows must submit a separate application to cover their salary.

DEADLINES AND INSTRUCTIONS FOR APPLICATIONS:

are available on the [AFM-Telethon website](https://www.afm-telethon.com).

[Access to the applicant portal](https://www.afm-telethon.com)

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