Postdoctoral researcher – Genome editing for inherited muscular disorders – Paris area, France

Description

A postdoctoral position is available in the therapeutic genome editing team under the direction of Dr. Mario Amendola in Genethon, biopark of Evry, France and it is funded by the Horizon Europe (https://magic-horizon.eu/).

This project deals with neuromuscular and musculoskeletal disorders, which are amongst the most frequent causes of disability in Europe. These genetic diseases are characterized by severe skeletal muscle wasting, impaired mobility, reduction of quality of life and premature death. Although supportive treatments are in clinical practice to manage symptoms, there is still no cure for muscular dystrophies. Thus, the overarching objective of our project is the generation of innovative models of human skeletal muscle disorders to develop, screen and validate new neuromuscular gene therapy vectors and genome editing strategies with high efficacy and selective tropism coupled with low toxicity and limited immunogenicity.

Relevant publications below.

Our laboratory focuses on: 1) developing effective and safe genome editing and gene therapy strategies for the treatment of human genetic diseases, in particular muscular disorders, hemoglobinopathies and lysosomal storage; 2) understanding of these pathologies and their molecular mechanisms; 3) understanding DNA repair and how to modulate/exploit to improve efficacy and safety of genome editing tools.

Qualifications

First and foremost, the desire to make an impact in the field of gene therapy in a friendly, enthusiastic, dedicated and collaborative manner.

Candidates should be able to work independently and interactively in a team setting, be responsible, organized and have a great work capacity and enthusiasm for research.

The candidate is expected to have a strong background in muscle biology, familiarity with molecular and cellular biology is a plus. Candidates should have at least one first author publication in a good quality journal and excellent communication skills in spoken and written English.

If you are strongly interested in this position but do not meet one or more criteria, please address in your cover letter in which aspects your skill set deviates from the outlined profile as well as how your background would enrich our research.

Host institute

Genethon (https://www.genethon.com/) is a non-profit biotherapy R&D organization dedicated to the development of gene therapy products for rare human genetic diseases. To this aim, Genethon partners with the university of Paris-Saclay and the INSERM research unit UMR_S951-INTEGRARE (http://integrare-umrs951.jimdo.com/), which hosts several laboratories exploiting gene-based technologies to investigate biological systems and pathologies of genetic origin and to design gene therapy treatments mainly for: i) blood and immune disorders, ii) neuromuscular disorders, iii) liver and metabolic disorders.

Genethon offers several high quality core facilities and infrastructure including:

- in vitro and in vivo (mice and rats) therapeutic testing platform: a functional evaluation platform (including ultrasound testing); an imaging-cytometry platform (confocal, macro confocal and biophotonic microscopy, imaging flow cytometry); a viral vector research facility (for LV, RV and AAV); tools and experience in molecular and physiopathological examination of isolated living cells; a histology department
- the largest DNA and cell bank in Europe for human genetic disorders

Details

- Salary: 56 800 €, according to experience
- 1 year full-time contract (renewable, based on performance)
- Starting date: January 2024 (negotiable).

How to apply

If your profile matches the description, please send an email to Dr. Mario Amendola (<u>mamendola@genethon.fr</u>). The application should include:

• a research interest letter

- a curriculum vitae
- at least two references (names and contact information)

Documents should be sent as a single pdf file no later than the 31st of October 2023

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Publication

1. Guiraud S, Dastidar S, Mazed F, Amor F, Ralu M, de Cian A, Richard I, Ronzitti G, Tedesco FS, Amendola M. CRISPR-Cas9 mediated endogenous utrophin upregulation improves Duchenne Muscular Dystrophy *BioRxiv*