

## *Postdoctoral researcher - hematopoietic stem cell based gene therapy – Paris area, France*

### *Description*

An INSERM postdoctoral position is available in the therapeutic genome editing team under the direction of Dr. Mario Amendola in Genethon, biopark of Evry, France

More than 300 patients have now been treated with HSPC gene therapy in clinical trials, and robust evidence for the durability of corrected HSPC treatments and their long-term safety and clinical efficacy has been demonstrated for several monogenic diseases. We plan to improve HSC engineering to: a) develop alternative ways to harness hematopoietic stem cells as a delivery vehicle for therapeutic proteins, thanks to the ability of their lineages to grow in many tissues, including the central nervous system; b) treat systemic disorder, such as lysosomal storage and blood clotting disorders.

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 hematopoietic stem cell biology and transplantation, 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 2023 (negotiable).

### *How to apply*

If your profile matches the description, please send an email to Dr. Mario Amendola ([mamendola@genethon.fr](mailto:mamendola@genethon.fr)).

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

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.

### *Publications*

1. Pavani G., Fabiano A., Laurent M., Amor F., Cantelli E., Chalumeau A., Concordet J.P., Mavilio F., Ferrari G., Miccio A., Amendola M.  
Correction of  $\beta$ -thalassemia by CRISPR/Cas9 editing of the  $\alpha$ -globin locus in human hematopoietic stem cells  
Blood Advances, 2021 Mar 9;5(5):1137-1153
2. Pavani G., Laurent M., Fabiano A., Cantelli E., Sakkal A., Corre G., Lenting P.J., Concordet J.P., Toueille M., Miccio A., Amendola M.  
Ex vivo editing of human hematopoietic stem cells for erythroid expression of therapeutic proteins  
Nat Commun. 2020 Aug 13;11(1):4146
3. Pavani G., Amendola M.  
Targeted gene delivery: where to land  
Front. Genome Ed., 2021 Jan 20;2:609650