Institute of Molecular Genetics of the Czech Academy of Sciences
invites applications for the following open position

1 PHD POSITION IN EXOSOME MEDIATED GENE THERAPY OF DIAMOND BLACKFAN ANEMIA FOR THE MSCA DOCTORAL NETWORKS PROJECT "GENE THERAPY OF RARE DISEASES" (GETRADI)

Starting date: March 1, 2023
Duration: 36 Months
EU research and innovation programme: Horizon Europe / Marie Skłodowska-Curie Actions (GRANT AGREEMENT NUMBER: 101072427)

Project title

Development of therapeutic exosomes and gene therapy for Diamond Blackfan Anemia (DBA)

The group and our research

The position is available in the Laboratory of Transgenic Models of Diseases, which closely cooperates with the Czech Centre for Phenogenomics (CCP; www.phenogenomics.cz), which is providing comprehensive expertise in in vivo research of gene functions using animal models, especially the full spectrum of genetic engineering, including generation of mouse models of diseases, advanced phenotyping and imaging techniques, and correction of a gene function to propose disease treatment.

Objectives

- Development of Extracellular vesicle (EV)-based and AAV-based gene therapy of hematopoietic stem cells (HSC) to rescue the Diamond-Blackfan Anemia (DBA) model; development of delivery/therapeutic protocols,
- In vitro/ex vivo correction of HSC using genetic approach and site-specific nucleases,
- In vivo analysis of DBA disease mouse model after the therapeutic approach.

Expected results

- Developed delivery therapeutic approaches based on AAV and EVs for correction of haematology disorders.

- Established an experimental approach for DBA gene therapy in a model system.

Work location and supervisor name

Laboratory of Transgenic Models of Diseases and Czech Centre for Phenogenomics, Institute of Molecular Genetics, BIOCEV campus, Prumyslova 595, 252 50 Vestec, Czech Republic

Supervisor
PD. Dr. rer. nat. habil. Radislav Sedláček

Required skills/qualification

We are looking for a highly motivated and enthusiastic candidate with the following qualifications:

- A Master of Science (or equivalent) degree in biology or biotechnology or similar relevant field obtained no later than on 28 February 2023.
- Experience in biochemistry, molecular biology, genetics and cell culture is expected
- General knowledge about biology, physiology and reproduction will be of advantage
- Excellent communication skills in English are requested

Project background and goal

More than 300 million people worldwide are suffering from more than 6000 rare diseases that are mostly caused by a single inherited mutation. Repair of the defective gene by gene editing is the only possible curative therapy. However, only for very few rare diseases such gene editing therapy has reached the clinic. The “Gene therapy of Rare Diseases” (GetRadi) consortium aims to contribute strongly to the establishment of more gene therapies for rare diseases by training the future leaders in gene therapy of rare diseases preforming ambitious projects with the following objectives:

1. Improving transfer of genome editing tools to target cells,
2. improving gene editing efficiency, and
3. improving safety of gene therapy.

Strong participation of the pharmaceutical industry to research projects and training, development of novel in vitro and in vivo models for rare diseases to test gene therapies in relevant settings, and application of several unique genome editing tools to treat rare diseases are the hallmarks of the GetRadi network.

Eligibility criteria

Applicants need to fully comply with these eligibility criteria:

1. The potential fellow must not be in possession of a doctoral degree at the date of recruitment. Researchers who have already successfully defended their doctoral thesis but have not yet been awarded their degree are not eligible.
2. Conditions of international mobility of researchers: The applicant must not have resided or carried out his or her main activity (work, studies, etc.) in the Czech Republic for more than 12 months in the 36 months immediately before the date of recruitment. Compulsory national service, short stays such as holidays, and time spent as part of a procedure for obtaining refugee status under the Geneva Convention are not taken into account.

How to apply

The job application in English consisting of a cover letter and structured CV should be sent to Mr. Marketa Morska (marketa.morska@img.cas.cz) **by 31 October 2022**. If you have any questions related to the positions, please send them to this email as well.

We thank all who apply for their interest; however, only those candidates who are considered for an interview will be contacted.

Additional information

The candidate is expected to enrol in a doctoral program from March 1, 2023.

The position is offered under the conditions of the Marie Skłodowska Curie Action Doctoral Network Scheme and therefore offers a salary that is determined by the regulations of this scheme. This equates the gross monthly salary 2,422 EUR (including Marie Skłodowska Curie mobility allowance) / 2,908 EUR (including family allowance, if applicable).

In addition to their individual scientific projects, all ESR will benefit from further continuing education, which includes scientific skills courses, transferable skills courses, as well as active participation in workshops and conferences and secondments to partner labs.

The European GetRadi training network wishes to reflect the diversity of society and welcome applications from all qualified candidates regardless of age, disability, gender, nationality, race, religion or sexual orientation.

Funded by
the European Union

GetRadi project has received funding from the European Union's Horizon Europe research and innovation programme under the Marie Skłodowska-Curie grant agreement No. 101072427