

IBEC-VHIR INTERNATIONAL PhD PROGRAMME

Position

1. Project Title/ Job Position title:
Development a Muscle-on-Chip Platform for LAMA2 Muscular Dystrophy Gene Therapy
2. Research project/ Research Group description

This project aims to develop an innovative Muscle-on-Chip (MoC) platform to advance gene therapy for LAMA2-related muscular dystrophy, a severe, early-onset, orphan neuromuscular condition caused by mutations in the LAMA2 gene. By collaborating with clinical partners (pediatric neuromuscular team), gene and cell therapy experts at VHIR, and the Biosensor for Bioengineering group at IBEC, this thesis integrates biomedical engineering, advanced biosensor technology, and gene therapy innovation to find a transformative therapy for affected children. The project is part of a dynamic international network with collaborations across Europe and the US.

Main Objectives:

Objective 1: Engineer a functional 3D skeletal muscle co-culture.

- **Task 1.1:** Obtain myoblasts and FAPs from patients' muscle biopsies and murine models. (VHIR)
- **Task 1.2:** Establish and characterize a novel 3D skeletal muscle co-culture model for LAMA2-related muscular dystrophy. (IBEC)

Objective 2: Develop the Muscle-on-Chip platform integrated with a plasmonic biosensor to monitor fibrosis related to LAMA2 deficiency.

- **Task 2.1:** Fabricate microfluidic devices for the MoC platform. (IBEC)
- **Task 2.2:** Characterize fibrosis markers in human cells and mouse models. (VHIR)
- **Task 2.3:** Develop multiplexing plasmonic biosensors for detecting fibrosis markers and LAMA2 protein. (IBEC)
- **Task 2.4:** Integrate and validate the MoC platform. (IBEC)

Objective 3: Test gene therapy on the MoC platform and validate it in a LAMA2-related dystrophy mouse model.

- **Task 3.1:** Design and test gene therapy for LAMA2 gene replacement or mutation editing in human cells. (VHIR)
- **Task 3.2:** Test gene therapy on the MoC platform. (IBEC)
- **Task 3.3:** Validate gene therapy in the LAMA2 mouse model. (VHIR)

3. Job position description

The Biosensors for Bioengineering group (IBEC) is seeking a looking for an Early-Stage Researcher to join as a PhD student to join our multidisciplinary project aimed at developing an advanced Muscle-on-Chip (MoC) platform. This platform will be used to study LAMA2-related muscular dystrophy and test novel gene and cell therapy approaches. This role offers an exciting opportunity to work at the intersection of muscle biology, tissue engineering, biosensor technology, and therapeutic development, contributing to cutting-edge advancements in personalized medicine. The PhD student will work in the Biosensors for Bioengineering group (IBEC) and the Gene and Cell Therapy group (VHIR) in collaboration with the Pediatric Neurology group (Vall de Hebron hospital). Both groups have experience in the topic and experience working together in other projects.

Main Tasks and Responsibilities:

- Develop and characterize 3D tissue co-cultures of skeletal muscle tissues from patient-derived cells.
- Design and fabricate microfluidic devices and biosensors.
- Design and test gene therapy for LAMA2 in cell culture.
- Integrate the platform to test gene therapy approaches for LAMA2.
- Test gene therapy in mouse models.

Requirements for Candidates:

Essential:

- Degree in Biology, Biomedicine, Biomedical Engineering, or a related field.
- Experience in cell culture and molecular biology.
- High level of English.
- Competencies and skills: Communication, teamwork and collaboration, commitment, proactivity, integrity, critical and analytical thinking.

Desirable:

- Experience in skeletal muscle research.
- Experience in muscular dystrophy research.
- Experience in microphysiological systems and tissue engineering.
- Experience in animal research.

Group Leader at IBEC

1. Title: ICREA Professor
2. Full name: Javier Ramón Azcón
3. Email: jramon@ibecbarcelona.eu
4. Research Group: Biosensors for Bioengineering

Group Leader at VHIR

1. Title: M.D. Senior Researcher
2. Full name: Jordi Barquinero Mániz
3. Email: jordi.barquinero@vhir.org
4. Institute: VHIR
5. Research group: Gene & Cell Therapy