

POSTDOC POSITION IN GENE THERAPY

The **EB House Austria** is an EU Reference Center of Excellence for the rare genetic skin disease Epidermolysis bullosa (EB), affiliated with the University Hospital Salzburg and the Paracelsus Medical University. We consist of four units – an outpatient unit, academy, research unit, and clinical study center – and adopt a holistic approach to developing and delivering outstanding multi-disciplinary care to patients with EB.

The group of Dr. Ulrich Koller is looking for postdoctoral candidates interested in developing gene therapies for EB. EB is a devastating disease characterised by diminished skin integrity due to mutations in genes encoding structural proteins necessary for maintaining proper adherence of the different skin layers to each other. Children with EB are also known as "butterfly children", a reference to the fragile nature of their skin, similar to a butterfly's wings.

Project

The project involves the development and improvement of a nuclease-based gene therapy for JEB via repair of a distinct mutation within the COL17A1 gene. Our main focus is on the efficiency and specificity of this applied technology. Despite the promise of programmable nucleases, the potential risk of off-target events is a recognized obstacle to clinical application. In this project we aim to achieve a potential traceless correction of the disease-causing mutation using the Cas9 mutant D10A (Cas9n), which is associated with higher safety profiles. The expression of COL17A1 and the restoration of functional collagen XVII protein in corrected cells will be analysed by sqRT-PCR, immunofluorescence and Western blot analysis. The proper deposition of the restored protein at the dermo-epidermal junction of the skin will be analysed in in vitro-generated 3D skin equivalents and mouse models. Furthermore, safety profiles for this gene editing application will be evaluated. Project Start: October 2020

Candidate

The ideal candidate should be highly motivated, reliable, flexible, able to work both independently and as part of a team, and have strong time-management and communication skills. Other qualifications include:

+ PhD (or equivalent) in molecular or cell biology, genetics, biotechnology or similar

- + Technical/laboratory experience in:
 - sqRT-PCR, cloning, immunofluorescence, western blotting
 - cell culture (isolation and cultivation of different cell types, 3D skin equivalents)
 - o basic flow cytometry including analysis
 - optional skills: gene-editing, animal experimentation
- + Track record of peer-reviewed publications
- + Fluent in written and spoken scientific English

Position

Full-time (40 hours/week) postdoc position, limited to a maximum of 3 years. Minimum salary according to FWF personnel cost unit rate: 54453 € brutto/year.

Working environment

The EB House Research unit consists of four collaborative groups dedicated to the development of life-enhancing therapies for patients. Our labs are well-equipped for the investigation of cells, genes, RNA, and proteins, including in-house and interdepartmental facilities for flow cytometry, NGS, quantitative real-time PCR, and microscopy. We have expertise in three-dimensional organotypic in vitro models. murine models. bioinformatics, and next generation sequencing platforms. The lab of Dr. Koller is situated on the campus of the Paracelsus Medical University.

Application

Please send your application (CV, motivation letter, and contact info of three references) to: **Dr. Ulrich Koller**, <u>u.koller@salk.at</u>. Deadline: 15/06/2020



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