5th Anniversary
CENTER FOR DERMAL RESEARCH

RUTGERS' CENTER FOR DERMAL RESEARCH
(CDR) SEMINAR SERIES

Guest speaker: **Joanna Jacków**

Rutgers, The State University of New Jersey
February 15, 2017 at 5:30pm

Gene-corrected fibroblast therapy for recessive dystrophic epidermolysis bullosa using a SIN COL7A1 retroviral vector

ABSTRACT:

Recessive dystrophic epidermolysis bullosa (RDEB) is a severe genetic skin fragility disorder caused by mutations in the COL7A1 gene encoding type VII collagen, which is the major constituent of anchoring fibrils (AF) at the basement membrane zone (BMZ). Patients with RDEB lack type VII collagen and therefore have severely impaired dermal-epidermal stability. There is currently no specific treatment for RDEB and several approaches using gene, cell and protein-based strategies are being developed. Here, we show that sub-cutaneous injections of gene-corrected RDEB dermal fibroblasts have the potential to reverse the disease phenotype in a human RDEB skin equivalent system in mice. We used a GMP-certified batch of a self-inactivating (SIN) COL7A1 retroviral vector and obtained 50 % transduction efficiency in primary RDEB fibroblasts. Transduced cells showed type VII collagen re-expression, displayed normal proliferative capabilities and viability, and improved adhesion properties in vitro. We provide evidence that intradermal injection of 3 x106 gene-corrected RDEB fibroblasts beneath RDEB skin equivalent grafted onto nude mice allows type VII collagen deposition at the dermal-epidermal junction and anchoring fibrils formation two months after treatment, supporting functional correction in vivo. These results provide a proof of principle that local injection of RDEB fibroblasts corrected with a GMP-certified SIN COL7A1 vector has a therapeutic potential for RDEB patients.

BIOGRAPHY:

About 3 years ago, I completed my PhD in the Department of Dermatology at the University Medical Center in Freiburg, Germany. Guided by a strong interest to pursue an academic carrier in the dermatology research field, I began my initial post-doctoral training in the Laboratory of Genetic Skin Diseases under the supervision of Prof. Alain Hovnanian in 2014 at the Imagine Institute in Paris, France. The post doctoral training experience convinced me that I would like to continue my scientific work in academia. Therefore, to extend my experience in the dermatology research area, I recently joined Prof. Christiano's lab in July 2016. From my 7 years of experience working on EB and skin biology, my overall career goal is to contribute to the fundamental understanding of inherited human skin diseases and develop effective therapeutic strategies using innovative technologies for treating skin disorders. I believe that this goal can be best achieved through combining molecular biology, cell culture and skin tissue engineering. Being in Prof. Christiano's lab, which has an internationally recognized-expertise in stem cell research and bioengineering, is an amazing environment to gain solid research experience and in perfect complementarity to my background in the field of dermatology. My future career goal is to obtain a position as an Assistant Professor to conduct my independent research.

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