

Institute for Regenerative Medicine (IREM) in collaboration with  
Wyss Translational Center Zurich (Wyss Zurich), Regenerative  
Medicine Technologies Platform

## Interdisciplinary Colloquium Regenerative Medicine I

**Tuesday, May 30, 2017 at 12:30 – 1:30 pm,  
Kleiner Hörsaal OST,  
University Hospital Zurich**

### **Prof. Roberto Speck**

**Department of Infectious Diseases and Hospital Epidemiology,  
USZ**

### **Gene Engineering an HIV Resistant Immune System**

We have a major effort in the laboratory to gene engineer an HIV resistant immune system. This work is done in collaboration with Prof. Karl-Heinz Krause (UNIGE, Geneva, CH) and Prof. Michael Pepper (University of Pretoria, South Africa).

For generating HIV resistant CD4+ T-cells, we have developed an optimized microRNA for down-regulating the HIV co-receptor CCR5. Using this microRNA we have gotten first-proof-of pre-clinical concept data that gene engineering HIV resistant CD4+ T-cells constrains HIV replication in vivo using humanized mice (functional cure). Briefly, we introduced the microRNA to CCR5 via lentiviral-based transduction into CD34+ cells and transplanted these gene-engineered CD34+ cells into the liver of new-born mice. 12 weeks later the humanized mice were infected with replication competent HIV.

We found that humanized mice expressing >50% of gene engineered CD4+ had HIV RNA copy numbers less than 5.000/μl – HIV-infected patients with such a low number of HIV RNA have a very protracted course of their HIV disease and in fact do not need any combined anti-retroviral treatment.

We are now working for identifying a 2nd anti-HIV target and getting all procedures/lab manipulations ready for a phase I clinical trial.

**Organiser:** Prof. Dr. Dr. Simon P. Hoerstrup

**Execution/Chair:** Dr. Steffen M. Zeisberger

IREM & Wyss Zurich, Univ. of Zurich and ETH Zurich