



University of
Zurich^{UZH}

Institute for Regenerative
Medicine (IREM)



Colloquium

Clinical Colloquium Regenerative Medicine

**Monday, 27th August 2018 at 1–2pm,
Institute for Regenerative Medicine (IREM),
University of Zurich, Wagistrasse 12,
WAD-904 (Founders Lab), 8952 Schlieren**

Prof. Dr. med. Janine Reichenbach

Centre for Primary Immunodeficiencies

University Children's Hospital Zurich

Gene Therapy – Past, Present and Future

Clinically encouraging implementation of viral vector mediated gene therapy started 16 years ago in patients with inherited severe immunodeficiency. The division of immunology at University Children's Hospital Zurich was one of the first and few highly specialized University medical centers worldwide to successfully complete a first-in-man clinical gene therapy trial for such an immunodeficiency: A gamma-retroviral vector was used to correct autologous hematopoietic stem cells (HSC) and cure children with an X-linked inborn defect of immune system phagocytes called chronic granulomatous disease (CGD).

Due to unexpected side effects with first generation gamma-retroviral gene therapy vectors in this and other clinical phase I/II trials viral vector based gene therapy subsequently went through a phase of intense refinement. Novel safety and efficiency improved viral vectors are now boosting the gene therapy field and are in the meanwhile available for a number of immune deficiencies, metabolic disorders, degenerative eye diseases, HIV, and malignant diseases.

The IREM-associated gene therapy group has developed a novel lenti-viral gene therapy vector for the autosomal-recessive form of CGD, which shall be translated into a first-in-man (clinical phase I/II) trial in 2019/20. Currently the group is working on innovative next generation gene therapy based on precise "gene surgery" via nucleases such as CRISPR-Cas9 for inherited immunodeficiencies. Another novel approach as opposed to viral mediated gene therapy, is protein-mediated genome modification, which we are developing as methodological platform for epigenetic editing.

In parallel, it is planned to join forces of basic scientists, physician scientists and clinicians to create a University Medicine Zurich based translational gene therapy platform. This flagship collaboration shall be exploited to advance and accelerate development and implementation of new gene and cell therapeutic modalities for other diseases with single gene defects such as metabolic or skin disorders and malignant diseases. In a second step, gene and cell therapies may also be developed for neurodegenerative diseases at IREM.

Organiser: Prof. Dr. Dr. Simon P. Hoerstrup / Prof. Dr. Roger M. Nitsch

Execution/Chair: Dr. Steffen M. Zeisberger / Dr. Christian Tackenberg

IREM, University of Zurich

www.irem.uzh.ch/en/teaching