

Meeting Program

Wednesday, 2008-10-08

Meeting Registration

Foyer of the MPI für Infektionsbiologie/DRFZ

14:00-18:30 Registration Desk in the Foyer of the MPI für Infektionsbiologie/DRFZ

Educational course

Paul Ehrlich Lecture Hall

Chairs: Toni Cathomen, Charité - Universitätsmedizin Berlin
Jens Bohne, Hannover Medical School

15:00 *RNAi: A novel therapeutic approach?*
V. Patzel, Max-Planck Institute for Infection Biology, Berlin

15:30 *Non-viral gene transfer*
B. Wittig, Free University of Berlin

16:00 *Viral vectors: Current developments and future perspectives*
S. Weger, Charité - Universitätsmedizin Berlin

16:30 *Imagination and imaging in immune and gene therapies*
J. Charo, Max-Delbrück-Center for Molecular Medicine, Berlin

17:00 *Coffee Break*

17:30 *Cross-talk immune system and stem cells*
H.-D. Volk, Charité - Universitätsmedizin Berlin

18:00 *Genetic engineering of designer T cells for adoptive therapy*
W. Uckert, Humboldt-University and Max-Delbrück-Center for Molecular
Medicine, Berlin

18:30 Meet the expert

19:30 *Speaker's Dinner*

Thursday, 2008-10-09

08:00-18:30 Meeting Registration

Foyer of the MPI für Infektionsbiologie/DRFZ

08:30 **Opening Remarks**

Paul Ehrlich Lecture Hall

Local Organizers

08:35 **Welcome Address**

*Karl Max Einhäupl, Chairman of the Executive Board,
Charité – Universitätsmedizin Berlin*

08:45 **Welcome Address**

*Ulrich Hengge, President DG-GT
Jens Bohne, Chair Study Group “Viral Vectors & Gene Therapy” of the GfV*

Technical developments I

Paul Ehrlich Lecture Hall

Chair: Stefan Weger, Charité - Universitätsmedizin Berlin

09:00 J. Keith Joung, Harvard Medical School, Boston, USA

*Engineering zinc finger nucleases for highly efficient modification of
endogenous human genes*

09:30 Len W. Seymour, Oxford University, UK

Adenovirus delivery and the innate immune system

10:00 Florian Kreppel, University of Ulm

(TD 1)

*Geneti-chemically modified adenovirus vectors carrying a large high-
affinity protein ligand on the hexon capsomer exhibit 20-fold increased
transduction of hepatocytes in vivo following intravenous vector
injection in mice*

10:15 Gabriele Lang, University Hospital Giessen and Marburg

(TD 2)

*Adenovirus-mediated expression of a sodium-iodid-symporter (NIS) in
neuroendocrine tumors (NET)*

10:30 *Coffee Break*

11:00 Kerstin Cornils, University of Frankfurt /Main

(PC 10)

*and University Medical Center Hamburg-Eppendorf
Marking of stem cells with promotor-deprived self-inactivating
gammaretroviral vectors*

11:15 Christian Brendel, Georg-Speyer-Haus, Frankfurt/Main

(TD 4)

*Lentiviral vectors for gene-therapy of x-cgd: specific and high
expression of gp91phox restricted to granulocytes*

11:30 Lydia J. Duerner, Paul-Ehrlich-Institute, Langen

(TD 5)

In vivo selection of retroviral display libraries for tumour homing

11:45 Thomas Schaser, Paul-Ehrlich-Institute, Langen

(TD 6)

*Highly efficient RNAi-mediated gene silencing in tumour tissue using
replication-competent retroviral vectors*

- 12:00 Verena Wally, Paracelsus Private Medical University, Salzburg (TD 7)
5' trans-splicing repair of the KRT14 gene
- 12:15 Denijal Kosovac, University of Regensburg (TD 9)
Modulation of long-time erythropoietin expression in mice by concerted modification of codon usage and intragenic CpG content
- 12:30 Award Ceremony “**DG-GT Forschungspreis**”, endowed by Pfizer
U. Hengge, H. Büning und Dr. J. Vollmer (Pfizer)
- 12:45 **Lunch Break and Poster Walk** *Foyer of the MPI für Infektionsbiologie/DRFZ*

Technical developments II

Paul Ehrlich Lecture Hall

- Chair: Hildegard Büning, University of Cologne
- 15:00 Richard O. Snyder, University of Florida, Gainesville, USA
rAAV vector manufacturing and product evaluation to support human clinical trials
- 15:30 Katharina Gellhaus, Charité - Universitätsmedizin Berlin (TD 10)
The fate of AAV vector genomes during DNA double strand break induced gene targeting
- 15:45 Stefan Weger, Charité - Universitätsmedizin Berlin (TD 11)
Development of single self-complementary AAV vector systems for Tetracycline-regulated expression of interfering RNA (RNAi) molecules
- 16:00 Stephan Märsch, University of Cologne (TD 12)
Stealth adeno-associated virus vectors identified by codon randomization of immunogenic epitopes
- 16:15 *Coffee Break*

Central nervous system

Paul Ehrlich Lecture Hall

- Chair: Regine Heilbronn, Charité - Universitätsmedizin Berlin
- 16:45 Matthew J. During, Ohio State University, Columbus, USA
Gene therapy in the brain: Have we passed a critical threshold where success is likely?
- 17:15 Günther Sperk, Medical University Innsbruck, Austria
Use of AAV vectors for identifying brain sites and mechanisms of emotionally driven behavior in mice
- 17:45 Felix Müller-Lerch, Charité - Universitätsmedizin Berlin (PC 1)
AAV vectors endorse efficient retrograde transduction of upper motor neurons in the CNS

- 18:00 Anke Schmidt, University of Rostock (PC 2)
Genetic engineering of endogenous adult neural stem cells in different brain regions by specific peptide-tagged Ad vectors: Implications for in situ therapy of neurodegenerative disorders
- 18:15 General Meeting DG-GT *Paul Ehrlich Lecture Hall*
- 19:30 *Social Event* *Hörsaalruine*

Friday, 2008-10-10

08:00-14:30 Meeting Registration *Foyer of the MPI für Infektionsbiologie/DRFZ*

T cells in gene therapy

Paul Ehrlich Lecture Hall

- Chair: Dorothee von Laer, Georg-Speyer-Haus, Frankfurt/Main
- 08:30 Richard A. Morgan, National Institutes of Health, Bethesda, USA
Current status of NCI TCR gene therapy trials
- 09:00 Reno Debets, Rotterdam, The Netherlands
TCR gene therapy to treat melanoma: Lessons from preclinical studies
- 09:30 Matthias Leisegang, Max-Delbrück-Center for Molecular Medicine, Berlin (PC 3)
T cell receptor gene-modified T cells with specificity against a broadly expressed renal cell carcinoma antigen
- 09:45 Wolfgang Koestner, Hannover Medical School (PC 4)
Evaluation of allogeneic versus autologous TCR gene transfer after hematopoietic stem cell transplantation
- 10:00 Daniel Sommermeyer, Max-Delbrück-Center for Molecular Medicine, Berlin (PC 5)
Designer T cell receptors to generate high avidity T cells for immunotherapy
- 10:15 Sebastian Newrzela, Georg-Speyer-Haus, Frankfurt/Main (PC 6)
Relative resistance of mature T cells to oncogene transformation
- 10:30 *Coffee Break*

Stem cells in gene therapy I

Paul Ehrlich Lecture Hall

- Chair: Ulrich Hengge, University of Düsseldorf
- 11:00 Meenhard Herlyn, The Wistar Institute, Philadelphia, USA
Farming cells to rebuild skin and melanoma

- 11:30 Wolfgang Walther, Max-Delbrück-Center for Molecular Medicine, Berlin (PC 7)
Phase I clinical trial for nonviral jet-injection gene transfer in patients with in-transit metastases from melanoma and cutaneous metastases from breast cancer
- 11:45 Patrick Schmidt, University of Cologne (PC 8)
Redirected T cells with an antibody-derived immunoreceptor to target melanoma
- 12:00 Ralf Wagner, University of Regensburg and Geneart AG, Regensburg (PC 9)
Development and immunogenicity of RNA- and codon-optimized HIV candidate vaccines in phase I clinical trials
- 12:15 Axel Schambach, Hannover Medical School (TD 3)
A modular lentiviral vector system for reprogramming of somatic cells into induced pluripotent stem (iPS) cells
- 12:30 Richard Gabriel, National Center for Tumor Diseases (NCT) and (PC 11)
German Cancer Research Center (DKFZ), Heidelberg
Design and performance of comprehensive integration site analysis in clinical gene therapy
- 12:45 **Lunch Break and Poster Walk** *Foyer of the MPI für Infektionsbiologie/DRFZ*

Stem cells in gene therapy II

Paul Ehrlich Lecture Hall

- Chair: Christopher Baum, Hannover Medical School
- 14:30 Stefan Karlsson, Lund University Hospital, Sweden
Stem cells and lysosomal storage disorders
- 15:00 Adrian J. Thrasher, University College London, UK
Gene therapy studies in severe combined immunodeficiency
- 15:30 Olga Kustikova, Hannover Medical School (PC 12)
Cell-intrinsic heterogeneity plays an important role in the development of clonal dominance after retroviral gene transfer into hematopoietic stem cells
- 15:45 Daniel C. Wicke, Hannover Medical School (PC 13)
Gene therapy of MPL deficiency: Evidence for successful correction of Mpl knock out mice
- 16:00 Stefan Stein, Georg-Speyer-Haus, Frankfurt/Main (PC 14)
Gene therapy for chronic granulomatous disease: Current status of the German clinical trial
- 16:15 **Poster Award** *Paul Ehrlich Lecture Hall*
- 16:30 End of Meeting