14th Annual Meeting DG-GT
&
4th Annual Workshop
"Viral Vectors and Gene Therapy" of the GfV

Heidelberg, July 18 to 20, 2007

Wednesday, 2007-07-18

15:00 pm – 19:00 pm	**PhD student course**
15:00 pm	**Opening Remarks and Chair** Christopher Baum *President DG-GT, Hannover Medical School,* *Dept. Experimental Hematology, Hannover, Germany*
15:15 pm	 Connie Eaves *Terry Fox Laboratory, BC Cancer Agency, Vancouver, Canada* **Targeting Hematopoietic Stem Cells for Gene Therapy** Genetic modification of hematopoietic stem cells offers opportunities for correcting a variety of inherited disorders and for creating disease models. Although much progress has been made, inadequate knowledge about the very nature of human hematopoietic stem cells continues to inhibit their effective targeting and expansion for clinical use. In this discussion, I will summarize some novel insights we have recently obtained from studies of hematopoietic stem cell regulation in mice. Understanding these may be critical in making progress in human hematopoietic stem cell based gene therapy strategies.
15:45 pm	 Jörg Vollmer *Discovery & Development, Coley Pharmaceutical GmbH, Düsseldorf, Germany* **Targeting the immune system via Toll-like receptors** Upon microbial infection, the host has to mount a multiplicity of immune responses via particular receptors, the Toll-like receptors (TLRs). The TLR9 sub-family detects pathogen DNA (TLR9) or RNA (TLR7/8), which can be mimicked by synthetic TLR agonists designed and developed by Coley Pharmaceutical. Administration of TLR agonists results in rapid activation of innate immunity followed by longer term adaptive responses. Coley designed distinct classes of TLR7,8,9 agonists and antagonists for targeted, disease-specific therapy. The CpG oligonucleotide PF-3512676 is in pivotal phase 3 registration trials in first-line non-small cell lung cancer. TLR agonists and antagonists represent immune modulatory drugs with broad potential applications in cancer, infectious diseases as well as autoimmune diseases.

16:15 pm	 Greg Towers *Department of Infection, UCL, London, UK* **The Antiviral Restriction Factor TRIM5alpha** TRIM5alpha has recently emerged as an important antiviral protein in mammals. Simian but not human TRIM5alpha can strongly block infection by human immunodeficiency virus type 1 (HIV-1). The mechanism of TRIM5alpha's antiviral activity remains incompletely solved but certain details have emerged. TRIM5alpha trimers target incoming retroviral capsids via their C terminal B30.2 domain, and in most cases, strongly block viral DNA synthesis by reverse transcription. TRIM5alpha is ubiquitinylated and rapidly turned over within cells although these properties do not appear to be essential for antiviral activity. TRIM5alpha is likely to have an important role in preventing viruses jump from one species to another and in protecting mammals against retroviral zoonotic infection.
16:45 pm	 Michael Boutros *Signaling and Functional Genomics, German Cancer Research Center, Heidelberg, Germany* **RNAi** A key advance in recent years has been the discovery of RNA interference (RNAi), which allows the silencing of genes through introduction of short double-stranded RNAs (dsRNAs). First described in C. elegans by Andrew Fire and Craig Mello in 1998, RNAi is now been widely used as a tool to silence gene expression both in model organisms and human cells. The session will cover different methods for RNAi in mammalian cells, how to design short-interfering (si) and short-hairpin (sh) RNAs, and how to manage potential pitfalls, such as off-target effects and low efficiencies.
17:15 pm – 17:30 pm	Coffee Break

17:30 pm	 David A. Williams *Division of Experimental Hematology, Cincinnati Children's Research Foundation, Cincinnati, OH, USA* **Rho GTPases in Stem Cell Localization and Retention** Rho GTPases are Ras-like molecules best studied fro their regulation of cytoskeletal functions controlling cell shape, adhesion and migration. Rho GTPases, particular Rac GTPases, have recently been implicated in hematopoietic stem cell functions. The session will provide an overview of Rho GTPase biochemistry and function and the role of Rac GTPases in hematopoietic stem cell engraftment and retention in the medullary cavity and during ontogeny.
18:00 pm – 19:00 pm	Meet the expert
19:00 pm	Speakers´ Dinner

Thursday, 2007-07-19

08:00 am	**Registration**
08:30 am	**Opening remarks** Christof v. Kalle *Conference Host, Director National Center for Tumor Diseases Heidelberg, Heidelberg, Germany*
08:40 am	**Welcome address** Christopher Baum *President DG-GT, Hannover Medical School, Dept. Experimental Hematology, Hannover, Germany*
08:45 am	**Welcome address** Toni Cathomen *Chair, Working Group Viral Vectors, Charité – Universitäts-medizin Berlin, Institute for Virology (CBF), Berlin, Germany*
08:50 am	**Heidelberg, Genes and Cancer** Otmar D. Wiestler *Chairman and Scientific Director, German Cancer Research Center, Heidelberg, Germany*
08:55 am	**Research for Patients** Rüdiger Siewert *Medical Director, University Hospital Heidelberg, Heidelberg, Germany*
09:00 am	**Surgical and Molecular Approaches to Pancreatic Cancer** Markus W. Büchler *Medical Director, Surgery University Hospital Heidelberg, Heidelberg, Germany*
09:05 am	**Stem Cells and Cancer** Anthony D. Ho *Medical Director, Division of Medical Clinic V (Haematology, Oncology, Rheumatology), University Hospital Heidelberg, Heidelberg, Germany*
09:10 am	**Plenary Talk** **Long live(d) Rac and Rho** David Williams *Division of Experimental Hematology, Cincinnati Children's Research Foundation, Cincinnati, OH, USA*
09:35 am	**Discussion**

09:40 am – 10:40 am	**Vector and Target Cell Interaction** **Chair** Hildegard Büning *Internal Medicine, Lab for AAV vector development, University Hospital of Cologne, Cologne, Germany* Axel Rethwilm *Institute for Virology and Immunobiology, University of Würzburg, Würzburg, Germany*
09:40 am	**Vector and Target Cell Interaction** **Surface** **Targeting the cell entry of enveloped viral vectors** Christian J. Buchholz *Section Gene Transfer Medicinal Products I, Division of Medical Biotechnology, Paul-Ehrlich-Institut, Langen, Germany*
09:55 am	**Discussion**
10:00 am	**Vector and Target Cell Interaction** **Cytoplasm** **Cellular Restriction of Retroviral Transduction** Greg Towers *Department of Infection, UCL, London, UK*
10:20 am	**Discussion**
10:25 am	**Vector and Target Cell Interaction** **Nucleus** **Emerging adenoviral hybrid vectors for somatic integration in small and large animal models** Anja Ehrhardt *Department of Virology, Max von Pettenkofer-Institute, Ludwig-Maximilians-University Munich, Munich, Germany*
10:40 am	**Discussion**
10:45 am – 11:15 pm	**Coffee Break and Posterwalk**
11:15 am – 12:15 pm	**Abstract Presentations – Vector development**
11:15 am	Title Name
11:25 am	**Discussion**
11:30 am	Title Name
11:40 am	**Discussion**

11:45 am	Title Name
11:55 am	**Discussion**
12:00 pm	Title Name
12:10 pm	**Discussion**
12:15 pm – 13:15 pm	**Vector development – DNA/RNA/Nonviral Transfer** **Chair** Hans-Georg Kräusslich *Hygiene-Institute, University Hospital Heidelberg, Heidelberg, Germany* Luigi Naldini, *San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan, Italy*
12:15 pm	**Vector development – DNA/RNA/Nonviral Transfer** **HIV and its interactions with the target cell** Hans-Georg Kräusslich *Hygiene-Institute, University Hospital Heidelberg, Heidelberg, Germany*
12:30 pm	**Discussion**
12:35 pm	**Vector development – DNA/RNA/Nonviral Transfer** **Integrating Vectors** **Novel approaches to regulate transgene expression and enhance the efficacy and safety of Gene Therapy** Luigi Naldini *San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan, Italy*
12:55 pm	**Discussion**
13:00 pm	**Vector development – DNA/RNA/Nonviral Transfer** **Non-Integrating Vectors** **Therapeutic modulation of pre-mRNA splicing by antisense derivatives of U7 small nuclear RNA** Daniel Schuemperli *Institute of Cell Biology, University of Bern, Bern, Switzerland*
13:20 pm	**Discussion**
13:25 pm – 14:30 pm	**Lunch Break and Posterwalk**
14:30 pm – 15:30 pm	**Abstract Presentations – Vector development**

14:30 pm	Title Name
14:40 pm	**Discussion**
14:45 pm	Title Name
14:55 pm	**Discussion**
15:00 pm	Title Name
15:10 pm	**Discussion**
15:15 pm	Title Name
15:25 pm	**Discussion**
15:30 pm – 17:45 pm	**Preclinical models** **Chair** Connie Eaves *Terry Fox Laboratory, BC Cancer Agency, Vancouver, Canada* Dorothee von Laer *Georg-Speyer-Haus, Institute for Biomedical Research, Applied Virology and Gene Therapy, Frankfurt, Germany*
15:30 pm	**Preclinical models** **Oncolytic Parvovirus H-1 as Cancer Therapeutics** Jean Rommelaere *Department of Tumor Virology and INSERM Cancer Virotherapy Unit, German Cancer Research Center, Heidelberg, Germany*
15:45 pm	**Discussion**
15:50 pm	**Preclinical models** **Virotherapy** **Oncolytic viruses – latest research findings in virotherapy** Stefan Kubicka *Division of Gastroenterology, Hepatology and Endocrinology, Hannover Medical School, Hannover, Germany*
16:05 pm	**Discussion**
16:10 pm – 16:45 pm	**Coffee Break and Posterwalk**

16:45 pm	**Preclinical models** **Cancer Gene Therapy** **Small regulatory RNAs in hematopoietic cells** Michaela Scherr *Division of Molecular Haematology, Hannover Medical School, Hannover, Germany*
17:00 pm	**Discussion**
17:05 pm	**Preclinical models** **Cancer Gene Therapy** **Cancer Target Identification by RNAi** Michael Boutros *Signaling and Functional Genomics, German Cancer Research Center, Heidelberg, Germany*
17:20 pm	**Discussion**
17:25 pm	**Preclinical models** **Cancer Gene Therapy** **Immunstimulatorische RNA: isRNA and 3pRNA** Gunther Hartmann *Director Department of Clinical Pharmacology, University Hospital Bonn, Bonn, Germany*
17:40 pm	**Discussion**
17:45 pm – 18:45 pm	**Abstract Presentation – Therapeutic Models**
17:45 pm	Titel Name
17:55 pm	**Discussion**
18:00 pm	Titel Name
18:10 pm	**Discussion**
18:15 pm	Titel Name
18:25 pm	**Discussion**
18:30 pm	Titel Name
18:40 pm	**Discussion**
18:45 pm – 19:30 pm	**General Meeting DG-GT and Posterwalk**
20:00 pm	**Social Event**

Friday, 2007-07-20

08:15 am – 11:10 am	**Translation** **<u>Chair</u>** Sunyoung Kim *Department of Biological Sciences, Seoul National University, Seoul, Korea* Monika Preuss *Genetics Science Safety & Regulation, Scientific Development and Bioethics Division, Department of Health Area 610, Wellington House, London, UK*
08:15 am	**Translation** **Production – Target Identification** **Considerations for the stable and transient production of retroviral vectors under cGMP** Andrea Schilz *CEO Eufets AG, Idar-Oberstein, Germany*
08:30 am	**Discussion**
08:35 am	**Translation** **Production** **Next Generation Sequencing** Bernhard Korn *Genomics & Proteomics Core Facilitiy, German Cancer Research Center, Heidelberg, Heidelberg, Germany*
08:50 am	**Discussion**
08:55 am	**Translation** **Production** **Integration site analysis in clinical samples** Manfred Schmidt *Division of Translational Oncology, National Center for Tumor Diseases (NCT) Heidelberg, Heidelberg, Germany*
09:10 am	**Discussion**
09:15 am	**Translation** **Production** **How to access antigen-specific lymphocytes for diagnosis and therapy** Alexander Scheffold *Immunomodulation, German Rheumatism Research Center (DRFZ), Berlin, Germany*
09:30 am	**Discussion**

09:35 am	**Translation** **Regulatory** **Development of New and Innovative Gene Medicines Based on Naked DNA and Retrovirus** Sunyoung Kim *Department of Biological Sciences, Seoul National University and ViroMed Co. Ltd., Seoul, Korea*
10:00 am	**Discussion**
10:05 am	**Translation** **GTAC / EMEA** **Approval of gene therapy trials in the UK and framework for driving translation forward** Monika Preuss *Genetics Science Safety & Regulation, Scientific Development and Bioethics Division, Department of Health Area 610, Wellington House, London, UK*
10:20 am	**Discussion**
10:25 am	**Translation** **AMG** **Clinical trial application for gene transfer medicinal products** Christian J. Buchholz *Section Gene Transfer Medicinal Products I, Division of Medical Biotechnology, Paul-Ehrlich-Institut, Langen, Germany*
10:40 am	**Discussion**
10:45 am	**Translation** **Diagnostic** **Challenges in Tumor Genetics Approached by Molecular Profiling** Peter Lichter *Division of Molecular Genetics, German Cancer Research Center, Heidelberg, Germany*
11:00 am	**Discussion**
11:05 am – 11:30 am	**Coffee Break and Posterwalk**
11:30 am	**Plenary Talk** **Breast Stem Cells: Lessons from the Blood** Connie Eaves *Terry Fox Laboratory, BC Cancer Agency, Vancouver, Canada*
11:55 am	**Discussion**

12:00 am – 14:40 pm	**Clinical Gene Therapy** **Chair** David Williams *Division of Experimental Hematology, Cincinnati Children's Research Foundation, Cincinnati, OH, USA* Ulrich Hengge *Department of Dermatology, University Hospital, Düsseldorf, Germany*
12:00 pm	**Clinical Gene Therapy** **Hematopoietic stem cell gene therapy for Wiskott-Aldrich-Syndrome** Christoph Klein *Department of Pediatric Hematology / Oncology, Hannover Medical School, Hannover, Germany*
12:15 pm	**Discussion**
12:20 pm	**Clinical Gene Therapy** **An Update on the Chronic Granulomatous Diseases Gene Therapy Study** Manuel Grez *Georg-Speyer-Haus, Institute for Biomedical Research, Applied Virology and Gene Therapy, Frankfurt, Germany* Marion Ott *University Hospital Frankfurt, Frankfurt, Germany*
12:40 pm	**Discussion**
12:45 pm	**Clinical Gene Therapy** **Live or let die: the case for chemoprotection and stem cell selection using gene transfer in malignant and genetic diseases** David Williams *Experimental Hematology, Cincinnati Children's Hospital, USA.*
13:00 pm	**Discussion**
13:05 pm – 14:00 pm	**Lunch Break and Posterwalk**
14:00 pm	**Clinical Gene Therapy** **Function of TCR gene modified T cells** Hans Stauss *University College London, Department of Immunology and Molecular Pathology, Royal Free Hospital, London, UK*
14:20 pm	**Discussion**

14:25 pm	**Clinical Gene Therapy** **From bench to bedside: development of TLR9 CpG oligonucleotide agonists** Jörg Vollmer *Coley Pharmaceutical GmbH, Düsseldorf, Germany*
14:40 pm	**Discussion**
14:45 pm – 15:45 pm	**Abstract Presentation – Therapeutic Models**
14:45 pm	Titel Name
14:55 pm	**Discussion**
15:00 pm	Titel Name
15:10 pm	**Discussion**
15:15 pm	Titel Name
15:25 pm	**Discussion**
15:30 pm	Titel Name
15:40 pm	**Discussion**
15:45 pm	**Poster Abstract Award**
16:00 pm	**Meeting adjourned**