nature medicine

VolkswagenStiftung

Genome Editing for Gene and Cell Therapy, a Herrenhausen Symposium

Herrenhausen Palace Conference Center
Hanover, Germany
November 3-4, 2016

Conference Program

Thursday, November 3

	7:30 a.m.	**Registration**
	8:55 a.m.	Welcome Remarks
Keynote	*Chair: Christine Borowski, Nature Medicine, USA*	
	9:00 a.m.	*Translating Genome Editing to the Clinic: Strategies, Considerations and Constraints* Toni Cathomen, The Medical Center – University of Freiburg, Germany
Session 1	**Genome editing** *Chair: Markus Elsner, Nature Biotechnology, Germany*	
	10:00 a.m.	*Altering, Improving, and Defining the Specificities of Crispr-Cas Nucleases* Benjamin Kleinstiver, Harvard Medical School, USA
	10:30 a.m.	*Manipulating the Mitochondrial Genome with Designer Nucleases* Carlos Moraes, University of Miami Miller School of Medicine, USA
	11:00 a.m.	**Coffee break**
	11:30 a.m.	*The Mechanisms of Precise Genome Editing Using Oligonucleotide Donors* Eric Hendrickson, University of Minnesota, USA
	12:00 p.m.	*From Surgery to Genome Surgery* Frank Buchholz, Technische Universität Dresden,Germany
	12:30 p.m.	**Lunch**
Panel	**Discussion on therapeutics** *Moderator: Christine Borowski, Nature Medicine, USA*	
	2:15 p.m.	Panelists: Katherine High, Spark Therapeutics, USA, Natalie Mount, Cell and Gene Therapy Catapult, UK Matthew Porteus, Stanford University School of Medicine, USA James Wilson, University of Pennsylvania, USA

Session 2	**Cell therapy (relevant to ex vivo applications)** *Chair: Alison Farrell, Nature Medicine, USA*	
	3:15 p.m.	*Therapeutic Genome Editing Using the Crispr/Cas9 System* Matthew Porteus, Stanford University School of Medicine, USA
	3:45 p.m.	*CAR T Cell Therapy: The CD19 Paradigm and Beyond* Isabelle Rivière, Memorial Sloan Kettering Cancer Center, USA
	4:15 p.m.	**Coffee break**
	4:45 p.m.	*Genome Editing for the Hemoglobin Disorders* Daniel Bauer, Harvard Medical School, USA
	5:15 p.m.	*Regulatory Considerations for Clinical Translation* Natalie Mount, Cell and Gene Therapy Catapult, UK
	5:45 p.m.	*CRISPR/CAS9 Protein Engineering for Cell Cycle-Specific Genome-Editing to Enhance Homology-Directed Repair* Tony Gutschner, MD Anderson Cancer Center, USA
	6:00 p.m.	**Reception**
	7:30 p.m.	Close of day

Friday, November 4

Keynote	*Chair: Christine Borowski, Nature Medicine, USA*	
	9:00 a.m.	*Genetic Engineering for T Cell Therapies* Carl June, University of Pennsylvania, USA
Session 3	**Delivery in general** *Chair: Markus Elsner, Nature Biotechnology, Germany*	
	10:00 a.m.	*Nucleic Acid Delivery Systems for RNA Therapy and Gene Editing* Daniel Anderson, Massachusetts Institute of Technology, USA
	10:30 a.m.	*Homologous DNA Recombination In Vivo with the Delivery of Cas9 Ribonucleoprotein and Donor DNA Complexed to Gold Nanoparticles* Niren Murthy, University of California, Berkeley, USA
	11:00 a.m.	**Coffee break**
	11:30 a.m.	*Novel rAAVs for Classical Gene Therapy and Genome Editing* Mark Kay, Stanford University School of Medicine, USA
	12:00 p.m.	*Gene Inactivation Using Crispr/Cas9: Implications for Gene Therapy of Dominant Genetic Disorders* Thierry VandenDriessche, Vrije Universiteit Brussel, Belgium

	12:30 p.m.	*Inhalable Nanoparticles for In Vivo Genome Editing Mediated by CRISPR-CAS9 Delivery for Undruggable KRAS Driven Lung Cancers* Aditi Mehta, Ludwig Maximilians University of Munich, Germany
	12:45 p.m.	**Lunch and Poster session**
Session 4	**Gene therapy (relevant to in vivo editing)** *Chair: Alison Farrell, Nature Medicine, USA*	
	2:45 p.m.	*AAV for Genome Editing of Liver Metabolic Diseases* James Wilson, University of Pennsylvania, USA
	3:15 p.m.	*Towards Clinical Translation of Targeted Gene Editing in Human Hematopoietic Stem Cells* Luigi Naldini, Ospedale San Rafaelle, Italy
	3:45 p.m.	*AAV-Mediated Gene Therapy for Genetic Disease* Katherine High, Spark Therapeutics
	4:15 p.m.	**Coffee break**
Session 5	**Biomedical applications** *Chair: Hannah Stower, Nature Medicine, USA*	
	4:45 p.m.	*Precise Disease Modeling Through Genome Editing* Lukas Dow, Weill Cornell Medical College, USA
	5:15 p.m.	*A CRISPR Dropout Screen Identifies Novel Therapeutic Targets in Acute Myeloid Leukaemia* Kosuke Yusa, Wellcome Trust Sanger Institute, UK
	5:45 p.m.	*Hit and Go CAS9 Delivered Through a Lentiviral Based Self-Limiting Circuit* Gianluca Petris, Università di Trento (CIBIO), Italy
	6:00 p.m.	Close of conference