

Genome Editing for Gene and Cell Therapy, a Herrenhausen Symposium

Herrenhausen Palace Conference Center

Hanover, Germany

November 3-4, 2016

Draft Conference Program

Thursday, November 3

- 7:30 a.m.** **Registration**
- 8:55 a.m. Welcome Remarks

Keynote

- 9:00 a.m. *Translating Genome Editing to the Clinic: Strategies, Considerations and Constraints*
Toni Cathomen, Universität Klinikum Freiburg, Germany

Session 1 Genome editing

- 10:00 a.m. *Defining, Altering, and Improving 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 Mechanism of Cas9/CRISPR Cleavase-, Nickase-, and Dual Nickase-Mediated Genome Editing in Human Somatic Cells*
Eric Hendrickson, University of Minnesota, USA
- 12:00 p.m. *From Surgery to Genome Surgery*
Frank Buchholz, Technische Universitäten Dresden, Germany
- 12:30 p.m.** **Lunch**

Panel

- 2:00 p.m. *Panel discussion on therapeutics*

Session 2 Cell therapy (relevant to ex vivo applications)

- 3:00 p.m. *Genome Editing of Hematopoietic Stem and Progenitor Cells*
Matthew Porteus, Stanford University School of Medicine, USA
- 3:30 p.m. *CAR T Cell Therapy : The CD19 Paradigm and Beyond*
Isabelle Rivière, Memorial Sloan Kettering Cancer Center, USA

Thursday, November 3 cont.

- 4:00 p.m. Coffee break**
Genome Editing for the Hemoglobin Disorders
 4:30 p.m. Daniel Bauer, Harvard Medical School, USA
- 5:00 p.m. *Regulatory Considerations for Clinical Translation*
 Natalie Mount, Cell and Gene Therapy Catapult, UK
- 5:30 p.m. Short talk
- 5:45 p.m. Conference dinner**
- 7:30 p.m. Close of day

Friday, November 4

Keynote

- 9:00 a.m. Carl June, University of Pennsylvania, USA

Session 3 Delivery in general

- 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. *Gene Editing In Vivo with Cas9 Ribonucleoprotein Complexed to Gold Nanoparticles*
 Niren Murthy, University of California, Berkeley, USA
- 11:00 a.m. Coffee break**
- 11:30 a.m. *Therapeutic Genome Editing without the use of Nucleases*
 Mark Kay, Stanford University School of Medicine, USA
- 12:00 p.m. *Efficient In Vivo Liver-Directed and iPS-Based Ex Vivo Gene Editing Using CRISPR/Cas9*
 Thierry VandenDriessche, Vrije Universiteit Brussel, Belgium
- 12:30 p.m. Short talk
- 12:45 p.m. Lunch and Poster session**

Session 4 Gene therapy (relevant to *in vivo* editing)

- 2:45 p.m. *AAV for Genome Editing of Liver Metabolic Diseases*
 James Wilson, University of Pennsylvania, USA
- 3:15 p.m. Luigi Naldini, Ospedale San Raffaele, Italy
- 3:45 p.m. *AAV-Mediated Gene Therapy for Genetic Disease*
 Katherine High, University of Pennsylvania, USA

Friday, November 4 cont.

4:15 p.m. **Coffee break**

Session 5 Biomedical applications

4:45 p.m. *Understanding Cancer Drivers Using Inducible In Vivo Genome Editing*
Lukas Dow, Weill Cornell Medical College, USA

5:15 p.m. *A CRISPR Dropout Screen Identifies Genetic Vulnerabilities in Acute Myeloid Leukaemia*
Kosuke Yusa, Wellcome Trust Sanger Institute, UK

5:45 p.m. Short talk

6:00 p.m. Close of conference