Precision Genome Engineering

January 8–12, 2017 | Beaver Run | Breckenridge, Colorado | USA

Scientific Organizers:
J. Keith Joung, Massachusetts General Hospital, USA
Emmanuelle Charpentier, Max Planck Institute for Infection Biology and Humboldt University, Germany and The Laboratory for Molecular Infection Medicine, Sweden
Olivier Danos, Biogen, USA

Precision genome engineering technologies enable targeted, highly efficient alteration of DNA sequences in living cells or organisms. Given their customizable nature, these technologies promise to be broadly useful both as biomedical research tools and as novel therapeutics for gene-based diseases. Given the rapidity with which this field moves, it is challenging for both newcomers and experts alike to stay updated with all of the latest advances. Furthermore, genome engineering necessarily encompasses a broad range of scientific expertise that includes fundamental mechanisms of DNA repair, basic science studies of bacterial-derived systems that form the basis of many of these technologies, innovations with genome engineering tool platforms, a wide variety of research applications spanning from model organisms to plants and mammalian cells, and pre-clinical and clinical studies aimed at translation into human therapeutics. The goal of this Keystone Symposia meeting is to bring together scientists from academia and industry with diverse but relevant expertise in a setting conducive to discussion of new results and potential collaborative efforts. Invited talks will cover a wide range of topics ranging from fundamental basic science through to clinical translation studies. Attendees of this meeting will have the opportunity to hear about the latest findings in this fast-paced field and to establish collaborations with scientists who have complementary expertise.

Session Topics:
- (Basic) Science of Recombination and DNA Repair
- Latest Advances in Genome-Editing Nuclease Technologies I & II
- Biology of CRISPR Systems
- Research Applications of Genome and Epigenome Engineering I & II
- Novel Technologies for Therapeutic Application of Genome Engineering
- Therapeutic Applications of Genome Engineering

*Scholarship Application & Discounted Abstract Deadline: **September 19, 2016***
*Abstract Deadline: **October 6, 2016***
*Discounted Registration Deadline: **November 9, 2016***

Note: Scholarships are available for graduate students and postdoctoral fellows and are awarded based on the abstract submitted.

Meeting Hashtag: #KSgenome
www.keystonesymposia.org/17A2

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Precision Genome Engineering (A2)
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Sponsored by Biogen, Editas Medicine, Inc., Merck & Co., Inc., Novo Nordisk A/S, Regeneron Pharmaceuticals, Inc., Sangamo BioSciences, Inc. and Thermo Fisher Scientific Inc.

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SUNDAY, JANUARY 8

Arrival and Registration

MONDAY, JANUARY 9

Welcome & Keynote Address

*__J. Keith Joung__, Massachusetts General Hospital, USA

__Luigi M. Naldini__, San Raffaele Telethon Institute, Italy
Precision Genome Engineering of Human Hematopoiesis for Treating Genetic Disease and Cancer

(Basic) Science of Recombination & DNA Repair

*__Emmanuelle Charpentier__, Max Planck Institute for Infection Biology, Humboldt University and The Laboratory for Molecular Infection Medicine Sweden, Germany

__David R. Liu__, Harvard University, USA
Base Editing: Genome Editing without Double-Stranded DNA Cleavage

__Maria Jasin__, Memorial Sloan Kettering Cancer Center, USA
DNA Repair Mechanisms and Genome Engineering

__Frederick W. Alt__, Boston Children's Hospital, USA
Recurrent DNA Break Cluster Genes in Neural Development, Diversification and Disease: Potential Analogies to Lymphocyte Rearrangement Processes

__Jean-Yves Masson__, Laval University Cancer Research Center, Canada
Short Talk: Regulation of the Tumor Suppressor PALB2: A Critical Player for Homologous Recombination at a CRISPR/Cas9-Induced DNA Double-Strand Break

__Katherine S. Pawelczak__, NERx Biosciences, USA
Short Talk: Chemical Enhancement of CRISPR/Cas9 Mediated Site-Specific Genome Engineering using Novel Inhibitors of the Ku-DNA Interaction

Workshop 1: In vivo Delivery of Genome Editing Nucleases

*__Olivier Danos__, Biogen, USA

Workshop 2: Ex vivo or in vitro Delivery of Genome Editing Nucleases

*__Luigi M. Naldini__, San Raffaele Telethon Institute, Italy

Latest Advances in Genome-Editing Nuclease Technologies I

*__J. Keith Joung__, Massachusetts General Hospital, USA

__Ralf Kuhn__, Max-Delbrueck Center, Germany
Stimulation of Homology-Directed Repair at CRISPR/Cas9-Induced Double-Strand Breaks

__Edward J. Rebar__, Sangamo BioSciences, USA
New Zinc Finger Nuclease Architectures for Precision Genome Engineering

__Jay Ashok Shendure__, University of Washington, USA
Applications of Genome Editing in Developmental Biology and Human Genetics

__Amit Choudhary__, Harvard Medical School, USA
Short Talk: Chemical Control of CRISPR-Cas9 in Cells and Organisms

Poster Session 1

TUESDAY, JANUARY 10

Latest Advances in Genome-Editing Nuclease Technologies II

*__Jennifer A. Doudna__, HHMI/University of California, Berkeley, USA

__Jonathan S. Weissman__, University of California, San Francisco, USA
Applications of CRISPR-Cas9-Based Gene Regulatory Proteins

__Andy May__, Caribou Biosciences, Inc., USA
DNA Repair Outcomes Following Cas9 Double-Stranded Breaks

__J. Keith Joung__, Massachusetts General Hospital, USA
Defining, Optimizing and Changing the Specificities of CRISPR-Cas Nucleases

__Sarah Jacobi__, Integrated DNA Technologies, USA
Short Talk: Efficient Homology-Directed Repair using Long Single-Stranded DNA Templates

__Channabasavaiah B. Gurumurthy__, University of Nebraska Medical Center, USA
Short Talk: Easi-CRISPR: A Simple and Efficient Method for Creating Reporter and Conditional Knockout Animal Models

__Fuqiang Chen__, MilliporeSigma, USA
Short Talk: Improvement of CRISPR Activity and Specificity via Proximal Binding of Multiple CRISPR/Cas Systems (proxy-CRISPR)

Workshop 3: Genome-Wide Specificities & Off-Target Effects of Genome Editing Nucleases

*__J. Keith Joung__, Massachusetts General Hospital, USA

Workshop 4: Design of Individual gRNAs and Genome-Wide Libraries of gRNAs for CRISPR-Cas Nucleases

*__John G. Doench__, Broad Institute of MIT and Harvard University, USA

Biology of CRISPR Systems

*__Maria Jasin__, Memorial Sloan Kettering Cancer Center, USA

__Emmanuelle Charpentier__, Max Planck Institute for Infection Biology, Humboldt University and The Laboratory for Molecular Infection Medicine Sweden, Germany
CRISPR-Cas9: An Ancient Bacterial Immune System Harnessed for Genome Engineering

__Eugene V. Koonin__, National Institutes of Health, USA
Discovery of Novel CRISPR-Cas Systems by Genome and Metagenome Sequence Database Mining and Evolution of Adaptive Immunity in Prokaryotes

__Joshua Keith Young__, Dupont Pioneer, USA
Short Talk: Identification and Characterization of Novel Cas9 Endonucleases

Poster Session 2

WEDNESDAY, JANUARY 11

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Research Applications of Genome & Epigenome Engineering I

*Barbara J. Meyer**, University of California, Berkeley, USA

Feng Zhang, Broad Institute of MIT and Harvard University, USA
Expanding the CRISPR-Cas Genome Engineering Toolbox

Angelo Lombardo, San Raffaele Telethon Institute for Gene Therapy, Italy
Therapeutic Applications of Epigenome Editing

Jennifer A. Doudna, HHMI/University of California, Berkeley, USA
Mechanism and Delivery of RNA-Guided Genome Engineering Proteins

Alister Funnell, Altius Institute for Biomedical Sciences, USA
Short Talk: Functional Characterization of Putative Regulatory Elements by Precision Genome Engineering

Robert J. Ihry, Novartis Institutes for BioMedical Research, USA
Short Talk: p53 Inhibition Enhances CRISPR/CAS9 Engineering in Human Pluripotent Stem Cells by Blocking DSB-Induced Toxicity

Shashank Patel, NCI, National Institutes of Health, USA
Short Talk: A Genome-Scale CRISPR Screen to Identify Essential Genes for T Cell Based Cancer Therapies

Research Applications of Genome & Epigenome Engineering II

*Angelo Lombardo**, San Raffaele Telethon Institute for Gene Therapy, Italy

Barbara J. Meyer, University of California, Berkeley, USA
Genome Editing of C. elegans and Other Non-Model Organisms

Daniel F. Voytas, University of Minnesota, USA
Precise Engineering of Plant Genomes

Andrea Crisanti, Imperial College London, UK
Building and Testing CRISPR-Based Gene Drives for Population Control in the Malaria Mosquito

Alex Marson, University of California, San Francisco, USA
Short Talk: Discovery of an Autoimmunity-Associated IL2RA Enhancer by Unbiased Targeting of Transcriptional Activation

Poster Session 3

THURSDAY, JANUARY 12

Novel Technologies for Therapeutic Application of Genome Engineering

*Olivier Danos**, Biogen, USA

Vic E. Myer, Editas Medicine, USA
Advancing CRISPR Technologies for Therapeutic Application

Daniel G. Anderson, Massachusetts Institute of Technology, USA
Nucleic Acid Delivery Systems for RNA Therapy and Gene Editing

James M. Wilson, University of Pennsylvania, USA
In vivo Genome Editing of Liver for Treating Metabolic Disease

Mark A. Kay, Stanford University, USA
rAAV-Mediated Genome Editing without the Use of Nucleases

Bin Li, Ohio State University, USA
Short Talk: Lipid-like Nanoparticles for mRNA Delivery in vivo

Knut Woltjen, CiRA, Kyoto University, Japan
Short Talk: Simultaneous Derivation of Disease-Relevant Point-Mutants and Concordant Isogenic Clones from Human Induced Pluripotent Stem Cells

Workshop 5: Ethics & Human Rights

*George J. Annas**, Boston University School of Public Health, USA
Why Precision Genome Engineering Should Not Be Used to (Try to) Make a "Better Baby"

Peter Dabrock, FAU Erlangen-Nürnberg, Germany
"Do Not Throw the Baby Out with the Bathwater" – Societal, Ethical and Governance Challenges of Precision Genome Engineering for Science and Society

Evelyne Shuster, Veterans Affairs Medical Center, USA
Editing the "Book of Life": Too Early to Prescribe?

Therapeutic Applications of Genome Engineering

*Mark A. Kay**, Stanford University, USA

Andrew M. Scharenberg, University of Washington, USA
Translational Genome Editing

Philippe N. Duchateau, Cellectis SA, France
TALEN®-Based Targeted Genome Modifications for Improved CAR T-Cell Adoptive Immunotherapy

Linhong Li, MaxCyte, Inc., USA
Short Talk: Mutation Correction of X-linked Chronic Granulomatous Disease

Sateesh Krishnamurthy, University of Iowa, USA
Short Talk: Correction of CFTR Splicing Mutation by Using CRISPR/Cas9 Genome Editing and Non-Homologous End Joining

Meeting Wrap-Up: Outcomes & Future Directions (Organizers)

FRIDAY, JANUARY 13

Departure