



# Precision Genome Editing with Programmable Nucleases

January 28–February 1, 2018 | Keystone Resort | Keystone, Colorado | USA

## Scientific Organizers:

**Jin-Soo Kim**, Seoul National University, South Korea

**Feng Zhang**, Broad Institute of MIT and Harvard University, USA

**Daniel F. Voytas**, University of Minnesota, USA

*Genome editing refers to the use of programmable DNA-targeting platforms to manipulate genetic and epigenetic information in living cells and organisms. Genome editing has been recently democratized by the development of RNA-guided endonucleases repurposed from CRISPR-Cas microbial defense systems against viruses or plasmids. Whereas novel tools such as Cpf1 and RNA-guided deaminases are being developed to expand the scope of genome editing, old tools such as ZFNs and TALENs are likely to undergo innovations as well. These tools enable genome-wide genetic screens in cell lines and genetic studies in both model and non-model organisms. Furthermore, genome editing holds great promise for broad applications in medicine and biotechnology, raising public interest and concerns. Yet genome editing is still limited by many factors such as inefficient delivery in vivo, off-target effects, and unwanted, alternative DNA repair. This conference offers opportunities to learn about new developments, advancements and applications in this fast-moving field.*

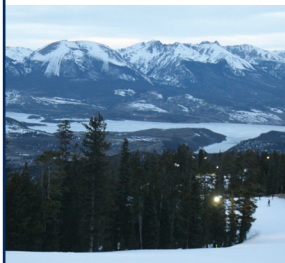
## Session Topics:

- DNA Repair and Genome Editing
- Genome Editing Methods and Novel Tools I & II
- Epigenome Editing and Gene Regulation
- Genetic Screening via CRISPR
- Therapeutic Genome Editing
- Plant and Animal Biotechnology
- Human Germline Editing and ELSI

**Scholarship Application & Discounted Abstract Deadline: October 3, 2017**

**Abstract Deadline: November 1, 2017**

**Discounted Registration Deadline: December 5, 2017**



Note: Scholarships are available for graduate students and postdoctoral fellows and are awarded based on the abstract submitted. Submitting an abstract is an excellent opportunity to gain exposure for your work. Abstracts submitted by the abstract deadline will also be considered for short talks on the program.

Meeting Hashtag: #KSgenome  
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# KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

## Precision Genome Editing with Programmable Nucleases (B1)

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Sponsored by AstraZeneca, Editas Medicine, Regeneron Pharmaceuticals, Inc., Sangamo Therapeutics, Inc. and Vertex Pharmaceuticals Incorporated

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### SUNDAY, JANUARY 28

#### Arrival and Registration

### MONDAY, JANUARY 29

#### Welcome and Keynote Address

\***Jin-Soo Kim**, Institute for Basic Science, South Korea

**Jennifer A. Doudna**, HHMI/University of California, Berkeley, USA  
*CRISPR Systems: Nature's Toolkit for Genome Editing*

#### DNA Repair and Genome Editing

\***Jacob E. Corn**, University of California, Berkeley, USA

**Maria Jasin**, Memorial Sloan Kettering Cancer Center, USA  
*Protecting the Genome by Homologous Recombination*

**Eric A. Hendrickson**, University of Minnesota Medical School, USA  
*The Mechanisms of Precise Genome Editing Using Oligonucleotide Donors*

**Eugene V. Koonin**, National Institutes of Health, USA  
*CRISPRICITY: A Metric for Measuring CRISPR Association and a Comprehensive Census of Cas Proteins*

**Chance Meers**, Georgia Institute of Technology, USA  
*Short Talk: Transcript-RNA from an Actively Transcribed Gene Accurately Repairs a DNA Double-Strand Break via a Homology-Driven Mechanism*

**Anastasia Lomova**, University of California, Los Angeles, USA  
*Short Talk: Improving Gene Editing by Temporal Control of DNA Repair*

#### NIH Somatic Cell Genome Editing Program: Overview

\***Elizabeth Wilder**, National Institutes of Health, USA

#### Workshop: Easi-CRISPR: CRISPRing Made Easier

\***C.B. Gurumurthy**, University of Nebraska Medical Center, USA

#### Genome Editing Methods and Novel Tools I

\***Daniel F. Voytas**, University of Minnesota, USA

**Feng Zhang**, Broad Institute of MIT and Harvard University, USA  
*Expanding the Genome Editing Toolbox by Harnessing Microbial Diversity*

**David R. Liu**, Broad Institute, HHMI, and Harvard University, USA  
*Base Editing: Chemistry on a Target Nucleotide in the Genome of Living Cells*

**J. Keith Joung**, Massachusetts General Hospital, USA  
*Efficient Gene Editing and Gene Regulation Using CRISPR-Cpf1 Nuclease Technology*

**Evan August Boyle**, Stanford University, USA  
*Short Talk: High-Throughput Biochemical Profiling of dCas9 Association and Dissociation Kinetics Uncovers New Rules for Off-Target Binding*

### Poster Session 1

### TUESDAY, JANUARY 30

#### Genome Editing Methods and Novel Tools II

\***Charles Gersbach**, Duke University, USA

**Scot A. Wolfe**, University of Massachusetts Medical School, USA  
*Orthogonal Cas9-Cas9 Fusions Provide a Versatile Platform for Precise Genome Editing*

**Jin-Soo Kim**, Institute for Basic Science, South Korea  
*Genome-Wide Target Specificities of CRISPR Nucleases and Deaminases*

**Jacob E. Corn**, University of California, Berkeley, USA  
*Discovery of Stimulation-Responsive Immune Enhancers with CRISPR Activation*

**Keiji Nishida**, Kobe University, Japan  
*Genome Editing with Non-Nuclease Editors from Bacteria to Plants*

**Alister Funnell**, Altius Institute for Biomedical Sciences, USA  
*Short Talk: High-Scale in situ Functional Mapping and Quantitative Engineering of Regulatory DNA*

**Omar Abudayyeh**, Massachusetts Institute of Technology, USA  
*Short Talk: Harnessing RNA Targeting CRISPR Systems for Transcriptome Engineering and Human Health*

#### Epigenome Editing and Gene Regulation

\***Scot A. Wolfe**, University of Massachusetts Medical School, USA

**Charles Gersbach**, Duke University, USA  
*Epigenome Editing for Gene Therapy, Cell Programming and Functional Epigenomics*

**Jennifer Mitchell**, University of Toronto, Canada  
*Uncovering Distal Regulatory Element Function Using CRISPR Genome Engineering*

**Joseph M. Miano**, University of Rochester, USA  
*Short Talk: CRISPR Interrogation of Regulatory Sequence Function in Mice*

**Chris Richardson**, University of California, Berkeley, USA  
*Short Talk: The Fanconi Anemia Pathway Plays a Key Role in Templated Repair at CRISPR-Cas9 Cut Sites*

### Poster Session 2

### WEDNESDAY, JANUARY 31

#### Genetic Screening via CRISPR

\***Edward J. Rebar**, Sangamo Therapeutics, Inc., USA

**Randall J. Platt**, ETH Zurich, Switzerland  
*CRISPR Screening Directly in vivo*

**Reuven Agami**, Netherlands Cancer Institute, Netherlands  
*Functional Genetic Screens of Regulatory DNA Elements*

**Neville Sanjana**, New York Genome Center & NYU, USA  
*New Frontiers for Pooled Screens: Finding Regulatory Elements in the Noncoding Genome and Capturing Multi-Cell Interactions*

**Danwei Huangfu**, Memorial Sloan Kettering Cancer Institute, USA  
*Human Development and Disease through the Lens of Pluripotent Stem Cells*

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**Bushra Raj**, Harvard University, USA

*Short Talk: Single-Cell Transcriptional Profiling Coupled with Lineage Tracing via Mutable Barcodes in the Vertebrate Brain*

**Jason D. Arroyo**, Pfizer, USA

*Short Talk: Using Human Genetics and Functional Genomics for a Locus-to-Gene Approach to Drug Target Validation*

### Panel: Somatic Cell Genome Editing Program: Discussion with NIH

\***Timothy LaVaute**, NINDS, National Institutes of Health, USA

### Therapeutic Genome Editing

\***Randall J. Platt**, ETH Zurich, Switzerland

**Edward J. Rebar**, Sangamo Therapeutics, Inc., USA

*Therapeutic Genome Editing and Gene Regulation Using Designed Zinc Finger Proteins*

**Amy J. Wagers**, Harvard University, USA

*In vivo Gene Editing in Muscle and Muscle Stem Cells*

**Charles F. Albright**, Editas Medicine, USA

*Development of a Subretinally-Delivered, CEP290-Specific CRISPR Medicine for the Treatment of Leber Congenital Amaurosis 10 (LCA10)*

**Pavitra Roychoudhury**, University of Washington, USA

*Short Talk: Rational Design and Evaluation of CRISPR/Cas9 Strategies for HIV Cure*

### Poster Session 3

### THURSDAY, FEBRUARY 1

#### Plant and Animal Biotechnology

\***Feng Zhang**, Broad Institute of MIT and Harvard University, USA

**Dan Carlson**, Recombinetics, Inc., USA

*Large Animal Genome Editing for Agriculture and Biomedicine*

**Caixia Gao**, Chinese Academy of Sciences, China

*Genome Editing with Programmable Nucleases in Crop Plants*

**Daniel F. Voytas**, University of Minnesota, USA

*Editing the Plant Genome*

**Weizhi Ji**, Kunming University of Science and Technology, China

*Primate Gene Editing and Human Complex Disease Study*

**Mariette E.S. Andersson**, Swedish University of Agricultural

Sciences, Sweden

*Short Talk: CRISPR-Cas9 Takes Several Bites in the Potato Genome- Efficient Targeted Multiallelic Mutagenesis in Tetraploid Solanum tuberosum*

**Asim Ahmad Beg**, University of Michigan, USA

*Short Talk: A Rapid and Facile C. elegans Genome Engineering Pipeline Using CRISPR/Cas9 Ribonucleoproteins*

#### Human Germline Editing and ELSI

\***Jin-Soo Kim**, Institute for Basic Science, South Korea

**Kathy K. Niakan**, Francis Crick Institute, UK

*Genome Editing Reveals a Role for OCT4 in Human Embryogenesis*

**Shoukrat Mitalipov**, Oregon Health & Science University, USA

*Human Germline Gene Correction*

**Tetsuya Ishii**, Hokkaido University, Japan

*The Potential Guidelines for Germline Genome Editing in Clinics*

### Meeting Wrap-Up: Outcomes and Future Directions (Organizers)

### FRIDAY, FEBRUARY 2

### Departure