# Keystone Symposia in Victoria

# Genome Engineering: From Mechanisms to Therapies

## Fairmont Empress Victoria | Victoria, British Columbia, Canada | February 19–23, 2019

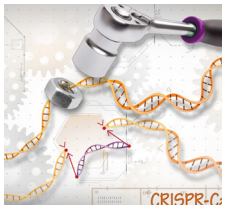
*Scientific Organizers:*
**Andrew May**, Chan Zuckerberg Biohub, USA
**Rodolphe Barrangou**, North Carolina State University, USA
**Knut Woltjen**, CiRA, Kyoto University, Japan

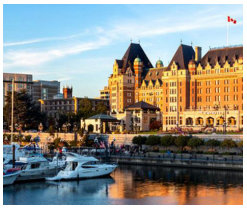
*The use of programmable nucleases such as CRISPR-Cas systems, ZFNs and TALENs has revolutionized cell biology by providing the ability to manipulate specific genetic and epigenetic states within living cells. These systems have been broadly applied as tools in research settings and increasingly are being used to develop improved models of disease and engineer cells for therapeutic purposes. Together with other DNA-modifying systems such as recombinases, integrases and transposases, it is now possible to introduce mutations that will model human disease, build complex synthetic signaling networks to perform regulated functions, and design cells to target specific disease states. Improvements to the methods involved requires understanding enzyme structures and mechanisms and how they intersect with cellular DNA repair systems. The intersection of this basic science with engineering approaches and improved cellular models is revolutionizing our understanding and treatment of human disease. The goal of this Keystone Symposia conference is to bring together those developing and studying genome engineering tools with groups who are applying them to build new disease models, identify disease mechanisms and drug targets, and develop cell-based therapeutics and genetic medicines. In addition to covering engineering of human and animal cells, this conference will also highlight the emerging field of genome engineering to identify new anti-microbial and anti-viral drugs and applications toward next-generation antibiotics. Invited talks will explore a broad range of topics covering new technologies, fundamental basic research, through the development of screening approaches, stem cell-based models of disease and design, and development of cellular therapeutics.*

*Plenary Session Topics:*
- Genome Engineering Tools and Technologies
- Workshop 1: Tools and Technologies
- Structure and Mechanism of Genome Editing Systems
- Engineered Models of Genetic Disease
- Workshop 2: Delivery
- Design and Engineering of Cellular Devices
- Genome Editing Screens for Function and Disease Mechanisms
- Harnessing DNA Repair Mechanisms for Genome Engineering
- Genome Engineering of Bacteria for Therapeutic and Diagnostic Applications
- Workshop 3: Clinical Trial Update
- Genome Editing for Treating Human Disease

*Abstract Deadline: Nov 20, 2018; Discounted Registration Deadline: Dec 19, 2018*

Visit **www.keystonesymposia.org/19B4** for more details.



KEYSTONE SYMPOSIA™
on Molecular and Cellular Biology
*Accelerating Life Science Discovery*
***a 501(c)(3) nonprofit educational organization***

# KEYSTONE SYMPOSIA
on Molecular and Cellular Biology

## Genome Engineering: From Mechanisms to Therapies (B4)

**February 19-23, 2019 • Fairmont Empress Victoria • Victoria, British Columbia, Canada**

**Scientific Organizers: Andrew May, Rodolphe Barrangou and Knut Woltjen**

*Sponsored by BlueRock Therapeutics, Editas Medicine and Thermo Fisher Scientific Inc.*

*Abstract & Scholarship Deadline: October 23, 2018 / Abstract Deadline: November 20, 2018 / Discounted Registration Deadline: December 19, 2018*

### TUESDAY, FEBRUARY 19

**Arrival and Registration**

### WEDNESDAY, FEBRUARY 20

**Welcome and Keynote Address**

**Philip D. Gregory**, Bluebird Bio, USA
*Talk Title to be Announced*

**Genome Engineering Tools and Technologies**

**Prashant Mali**, University of California, San Diego, USA
*Therapeutic Strategies via Genome Engineering: New Approaches and New Challenges*

**Zoltan Ivics**, Paul Ehrlich Institute, Germany
*Transposons: Molecular Parasites Tamed for Advanced Genome Engineering*

**Alexis C. Komor**, University of California, San Diego, USA
*Early Career Talk: Using Uracil as a Genome Editing Intermediate*

**Short Talk(s) Chosen from Abstracts**

**Workshop 1: Tools and Technologies**

**Short Talks Chosen from Abstracts**

**Structure and Mechanism of Genome Editing Systems**

**Benjamin P. Kleinstiver**, Massachusetts General Hospital, USA
*Engineered CRISPR Nucleases to Enhance Genome Editing*

**Andrew May**, Chan Zuckerberg Biohub, USA
*DNA Repair Outcomes Provide Insight into Genome Editing Mechanisms in Primary Cell Systems*

**Osamu Nureki**, University of Tokyo, Japan
*Structure and Mechanism of RNA-Programmable Nucleases*

**Short Talk(s) Chosen from Abstracts**

**Poster Session 1**

### THURSDAY, FEBRUARY 21

**Engineered Models of Genetic Disease**

**Danwei Huangfu**, Memorial Sloan Kettering Cancer Institute, USA
*Human Pluripotent Stem Cells as Models for Genetic Disease*

**Knut Woltjen**, CiRA, Kyoto University, Japan
*Isogenic Models of Disease from Induced Pluripotent Stem Cells*

**Amy J. Wagers**, Harvard University, USA
*Gene Editing in Stem Cells*

**Erika Sasaki**, Central Institute for Experimental Animals, Japan
*Talk Title to be Announced*

**Short Talk(s) Chosen from Abstracts**

**Poster Session 2**

**Workshop 2: Delivery Methods**

**Short Talks Chosen from Abstracts**

**Design and Engineering of Cellular Devices**

**Wilson Wong**, Boston University, USA
*Synthetic Biology in Cellular Immunotherapy*

**Timothy K. Lu**, Massachusetts Institute of Technology, USA
*Synthetic Gene Circuits for Next-Generation Therapeutics*

**Charles Gersbach**, Duke University, USA
*Cell Programming with Genome Engineering Technologies*

**Short Talk(s) Chosen from Abstracts**

### FRIDAY, FEBRUARY 22

**Genome Editing Screens for Function and Disease Mechanisms**

**Fyodor D. Urnov**, Altius Institute for Biomedical Sciences, USA
*Editing Human Genome Control Circuits to Reveal Disease Mechanisms and Targets for Intervention in the Clinic*

**Jan E. Carette**, Stanford University, USA
*CRISPR-Cas Screens for Studying Virus-Host Interactions*

**Speaker to be Announced**

**Brittany Adamson**, University of California, San Francisco, USA
*Early Career Talk: Perturb-Seq: Combining High throughput CRISPR Screening with Single-Cell Sequencing*

**Short Talk(s) Chosen from Abstracts**

**Harnessing DNA Repair Mechanisms for Genome Engineering**

**Maria Jasin**, Memorial Sloan Kettering Cancer Center, USA
*Homologous Recombination and End-Joining Mechanisms in Genome Editing*

**Beeke Wienert**, UC Berkeley, USA
*Unbiased Identification of CRISPR Off Targets in vivo using DISCOVER-seq*

**Nancy Maizels**, University of Washington School of Medicine, USA
*Gene Correction at Targeted DNA Breaks*

**Short Talk(s) Chosen from Abstracts**

**Poster Session 3**

### SATURDAY, FEBRUARY 23

**Genome Engineering of Bacteria for Therapeutic and Diagnostic Applications**

**Rodolphe Barrangou**, North Carolina State University, USA
*Engineered Lactobacilli for Human Health Applications*

**Jason M. Peters**, University of Wisconsin-Madison, USA
*Bacterial CRISPRi Screens to Identify the Mode of Action of Novel Antibiotics*

**Richard P. Novick**, New York University, USA
*Conversion of Staphylococcal Pathogenicity Islands (SaPIs) to CRISPR-Cas9-Carrying Antibacterial Drones That Cure Staphylococcal Infections*

**Short Talk(s) Chosen from Abstracts**

**Workshop 3: Clinical Development Progress**

**Short Talks Chosen from Abstracts**

**Genome Editing for Treating Human Disease**

**Bruce R. Conklin**, Gladstone Institutes, USA
*Genome Surgery for Treating Neuronal Disorders*

**Paula M. Cannon**, University of Southern California, Keck School of Medicine, USA
*Genome Editing to Treat Hematopoietic Disorders and Infectious Diseases*

**Michel Sadelain**, Memorial Sloan Kettering Cancer Center, USA
*Genome Edited CAR-T Cells for Improved Treatment of Solid Tumors*

**Juan Carlos Izpisua-Belmonte**, The Salk Institute for Biological Studies, USA
*Engineering Stem Cells in vivo for Therapeutic Benefit*

**Short Talk Chosen from Abstracts**

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

**SUNDAY, FEBRUARY 24**

**Departure**