

# KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

## Precision Genome Engineering (X2)

Scientific Organizers: Laura Sepp-Lorenzino and Matthew Porteus

Sponsored by (Cell Research), Chinese Society for Cell Biology, Synthego and Ultragenyx Pharmaceutical Inc.

## Genomic Instability and DNA Repair (X1)

Scientific Organizers: Katharina Schlacher, Irene Chiolo and Ralph Scully

March 19-23, 2023 • Fairmont Chateau Whistler • Whistler, BC, Canada

Sponsored by (Cell Research), Chinese Society for Cell Biology and Pfizer Inc.

Scholarship Deadline: December 14, 2022 / Abstract Deadline: December 14, 2022 / Discounted Registration Deadline: January 18, 2023

### SUNDAY, MARCH 19

#### Arrival and Registration

### MONDAY, MARCH 20

#### Welcome and Keynote Session (Joint)

##### Speaker to be Announced

**Titia de Lange**, Rockefeller University, USA  
*APOBEC3-Dependent Kataegis and TREX1-Driven Chromothripsis During Telomere Crisis*

#### Genome Instability and Cancer (Joint)

**Tanya T. Paull**, University of Texas at Austin, USA  
*Regulation of DNA Double-Strand Break Repair and Oxidative Stress Signaling*

**Simon J. Boulton**, Francis Crick Institute, UK  
*DNA Repair Mechanisms in Cancer and Therapy Approaches*

**Stephen C. West**, Francis Crick Institute, UK  
*DNA Repair – Structures and Mechanisms*

##### Short Talks Chosen from Abstracts

#### Roundtable Discussion: Genome Editing Ethics (X2)

#### Workshop 1: The Past and Future of DNA Repair/Replication (X1)

##### Short Talks Chosen from Abstracts

#### Natural Editing and Repair Mechanisms (X2)

**Toni Cathomen**, University Medical Center Freiburg, Germany  
*Quantitative Evaluation of Chromosomal Rearrangements in Gene-Edited Human Stem Cells by CAST-Seq*

**Anna Kwilas**<sup>†</sup>, Food and Drug Administration, USA  
*Regulatory Approach for Gene Editing: CBER Perspective*

##### Speaker to be Announced

##### Short Talks Chosen from Abstracts

#### Fork Protection and Cancer Therapy (X1)

**Alessandro Vindigni**, Washington University, USA  
*PRIMPOL-Mediated Adaptive Response Suppresses Replication Fork Reversal in BRCA-Deficient Cells*

**Sharon B. Cantor**, University of Massachusetts Medical School, USA  
*Replication Gaps Underlie BRCA Deficiency and Therapy Response*

**Li Lan**, Massachusetts General Hospital, USA  
*Understanding and Targeting R-Loops and mRNA-Dependent DNA Repair in Cancer*

##### Short Talks Chosen from Abstracts

#### Poster Session 1

### TUESDAY, MARCH 21

#### Plant and Animal Science Gene Editing Applications and Regulatory Path (X2)

**Catherine Feuillet**, Inari Agriculture, USA  
*SEEDesign Gene Editing: Gateway to New Future of Plant Breeding*

**Rodolphe Barrangou**, North Carolina State University, USA  
*CRISPR-Cas and Food: Implications for the Future*

**Caixia Gao**, Chinese Academy of Sciences, China  
*Applications of CRISPR-Cas in Agriculture and Plant Biotechnology*

**Irina A. Polejaeva**, Utah State University, USA  
*Advancing Large Animal Model Development using CRISPR/Cas 9 Editing*

##### Short Talks Chosen from Abstracts

#### Nuclear and Chromatin Dynamics in DNA Repair (X1)

**Gaëlle Legube**, Université Paul Sabatier, France  
*Chromosome Reorganization During DNA Double Strand Break Repair*

**Irene Chiolo**, University of Southern California, USA  
*DNA Repair Mechanisms in Heterochromatin*

**Matthias Altmeyer**, University of Zurich, Switzerland  
*Dynamics and Functions of DNA Repair Compartments*

**Karim Mekhail**, University of Toronto, Canada  
*Nuclear Dynamics and Phase Separation for DSB Repair in Yeast*

##### Short Talks Chosen from Abstracts

#### Workshop: Career Development (X2)

#### Workshop 2: New Approaches and Technologies to Study Genome Stability (X1)

##### Short Talks Chosen from Abstracts

#### Delivery of Gene Editors: Potency, Biodistribution, Safety (X2)

**Daniel J. Siegwart**, University of Texas Southwestern Medical Center, USA

*Selective Organ Targeting (SORT): A Strategy for Effective Tissue Specific mRNA Delivery and CRISPR/Cas Gene Editing*

**Charles Gersbach**, Duke University, USA  
*Long-Term Evaluation of AAV-CRISPR Genome Editing and Immunogenicity in in vivo Models*

**Jennifer E. Adair**<sup>†</sup>, Fred Hutchinson Cancer Research Center, USA  
*Nonviral Methods for Cell Engineering*

##### Short Talks Chosen from Abstracts

#### Inflammation and Genomic Instability (X1)

**Karl-Peter Hopfner**, Ludwig-Maximilians-University Munich, Germany  
*Structures of MRE11 and cGAS*

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**Katharina Schlacher**, MD Anderson Cancer Center, USA  
*Mitochondrial and Nuclear BRCA/FANC DNA Replication Stability in Disease and Cancer*

**Roger Greenberg**, University of Pennsylvania, USA  
*Genome Instability and Inflammation*

**Short Talks Chosen from Abstracts**

### Poster Session 2

### WEDNESDAY, MARCH 22

#### Genome Editing and DNA Repair (Joint)

**Maria Jasin**, Memorial Sloan Kettering Cancer Center, USA  
*Genome Modification by Natural and Artificial DNA Breaks*

**Jennifer A. Doudna**, HHMI/University of California, Berkeley, USA  
*The CRISPR Revolution: The Power and Promise of Gene Editing*

**Daniel Durocher**, Lunenfeld-Tanenbaum Research Institute, Canada  
*A Genetic Map of the Response to DNA Damage in Human Cells (CRISPR/Cas9 Screens)*

**David Pellman**, Dana-Farber Cancer Institute, USA  
*Chromothripsis*

**Short Talks Chosen from Abstracts**

#### Panel Discussion: Addressing COVID-19-Related Challenges for Women in Science (X1)

#### Editing Technology Development (X2)

**Nicole Gaudelli**, Beam Therapeutics, USA  
*Adenine and Cytosine Base Editors for the Treatment of Disease*

**Alex Marson**, University of California, San Francisco, USA  
*A Functional CRISPR Dissection of Gene Networks Controlling Human Regulatory T Cell Identity*

**Cecilia Cotta-Ramusino**, Tessera Therapeutics, USA  
*Gene Writing: A New Genome Engineering Technology*

**Short Talks Chosen from Abstracts**

#### Replication Coupled Repair (X1)

**Helle D. Ulrich**, Institute of Molecular Biology, Germany  
*New Insights into Ubiquitin-Dependent DNA Damage Bypass*

**Xiaolan Zhao**, Memorial Sloan Kettering Cancer Center, USA  
*Integrative Analysis Reveals Unique Structural and Functional Features of the Smc5/6 Complex*

**Ralph Scully**, Beth Israel Deaconess Medical Center, USA  
*Stalled Fork Repair in Health and Disease*

**Short Talks Chosen from Abstracts**

### Poster Session 3

### THURSDAY, MARCH 23

#### Ex vivo Gene Edited Cell Therapies (X2)

**Fyodor D. Urnov**, University of California, Berkeley, USA  
*From N=1 to N=all: a Platform Approach to CRISPR-Cas Therapies for Disorders of Hematopoiesis*

**Natalia Gomez-Ospina**, Stanford University, USA  
*Engineering the Blood to Treat the Brain Genome Editing*

**Chrystal Louis**, CRISPR Therapeutics, USA  
*CTX001: Pivotal Studies for Sickle Cell Disease and beta Thalassemia*

**Matthew Porteus**, Stanford University School of Medicine, USA  
*Sickle Cell Disease Clinical Program*

**Short Talks Chosen from Abstracts**

#### RNA Functions in Genome Stability (X1)

**Marco F. Foiani**, Istituto FIRC di Oncologia Molecolare, Italy  
*Role of ATR in Preservation of Cell Mechanics and Nuclear Integrity During Interstitial Migration*

**Karlene A. Cimprich**, Stanford University, USA  
*RNA Meets DNA: Dangerous Liaisons in the Genome*

**Fabrizio d'Adda di Fagnana**, IFOM, The FIRC Institute of Molecular Oncology, Italy  
*RNA Synthesis at DNA Lesions*

**Francesca Storici**, Georgia Institute of Technology, USA  
*Role of RNAs as Templates for DSB Repair*

**Short Talks Chosen from Abstracts**

#### In vivo Gene Editing Drug Development (X2)

**Laura Sepp-Lorenzino**, Intellia Therapeutics, USA  
*Clinical Results for In Vivo Knockout and Insertion CRISPR/Cas9 Programs*

**Mark S. Shearman**, Editas Medicine, USA  
*Clinical Progress on in vivo Gene Editing for Inherited Retinal Disorders*

**Sekar Kathiresan**, Verve Therapeutics, USA  
*Clinical Programs of Base Editors for Cardiovascular Disease*

**Short Talks Chosen from Abstracts**

#### Genomic Instability and Cancer 2 (X1)

**André Nussenzweig**, NCI, National Institutes of Health, USA  
*Talk Title to be Announced*

**Jan Karlseder**, The Salk Institute for Biological Studies, USA  
*Autophagic Cell Death Restricts Chromosomal Instability During Replicative Crisis*

**Agnel Sfeir**, Memorial Sloan Kettering Cancer Center, USA  
*Single-Molecule Imaging of Telomerase RNA Reveals a Recruitment-Retention Model for Telomere Elongation*

**Short Talks Chosen from Abstracts**

**Meeting Wrap-Up: Outcomes and Future Directions (Organizers) (X2)**

**Meeting Wrap-Up: Outcomes and Future Directions (Organizers) (X1)**

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**FRIDAY, MARCH 24**

**Departure**