

# KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

## Engineering the Genome (Q2)

Scientific Organizers: Vic Myer and Erik Sontheimer

Sponsored by Editas Medicine and Novo Nordisk A/S

## Emerging Cellular Therapies: Cancer and Beyond (Q1)

Scientific Organizers: Crystal L. Mackall, Marina Cavazzana and Stanley R. Riddell

February 8-12, 2020 • Fairmont Banff Springs • Banff, AB, Canada

Sponsored by BioLegend, Inc., Novo Nordisk A/S and Roche

Abstract & Scholarship Deadline: October 29, 2019 / Abstract Deadline: November 6, 2019 / Discounted Registration Deadline: December 10, 2019

### SATURDAY, FEBRUARY 8

#### Arrival and Registration

### SUNDAY, FEBRUARY 9

#### Welcome and Keynote Session (Joint)

**Carl H. June**, University of Pennsylvania, USA

*Updates with Engineered T Cells*

**Jennifer A. Doudna**, HHMI/University of California, Berkeley, USA

*Genome Editing with CRISPR-Cas Systems: Challenges and Opportunities in a New Era of Biology*

#### Biochemistry, Biophysics and New Enzymes (Q2)

**Kira S. Makarova**, National Center for Biotechnology Information, USA

*In silico Discovery of New Defense Systems*

**Ilya J. Finkelstein**, University of Texas at Austin, USA

*Massively-Parallel Profiling of Natural and Engineered High-Fidelity CRISPR Nucleases*

**Yanli Wang**, Chinese Academy of Sciences, China

*Structure Function of CRISPR Enzymes*

Short Talks Chosen from Abstracts

#### Genetically Engineered T Cell Therapies for Cancer: Results from the Clinic (Q1)

**Stanley R. Riddell**, Fred Hutchinson Cancer Research Center,

University of Washington, USA

*CAR T Cells for Solid Tumors*

**Christine E. Brown**, Beckman Research Institute, City of Hope, USA

*CAR T Cells for Brain Tumors*

**Kristen Hege**, Celgene, USA

*Clinical Development of BCMA Targeted Immunotherapies in Multiple Myeloma*

Short Talks Chosen from Abstracts

#### Workshop 1: Ethics of Genome Editing (Q2)

Short Talks Chosen from Abstracts

#### Workshop 1: Platforms for Immune Cell Engineering (Q1)

Short Talks Chosen from Abstracts

#### In Vivo Genome Editing (Q2)

**James M. Wilson**, University of Pennsylvania, USA

*In vivo Editing to Primate Liver*

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

*Update on Genome Editing in the Liver*

**David V. Schaffer**, University of California, Berkeley, USA

*Engineering and Evolving New Viruses for Gene Therapy and*

*Genome Editing*

Short Talks Chosen from Abstracts

#### Engineered Hematopoietic Stem Cells for Treatment of Genetic Diseases (Q1)

**Philippe Leboulch**, University of Paris-Sud and Harvard University, France

*Gene Therapy for Hemoglobinopathies: Challenges to Solve*

**Marina Cavazzana**, Paris Descartes University, France

*Gene Therapy for Immunodeficiencies*

**Alessandra Biffi**, Dana-Farber Cancer Institute, Boston Children's Hospital, USA

*Engineered HSCs for Correction of CNS Disorders*

Short Talks Chosen from Abstracts

#### Poster Session 1

### MONDAY, FEBRUARY 10

#### Genome Editing as a Biology Discovery Tool (Q2)

**Alan Huang**, Tango Therapeutics, USA

*Novel Therapeutic Opportunities Identified by Synthetic Lethal Screening*

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

*Towards an understanding of human development using gene ablation*

**Philipp Junker**, Max Delbrück Center for Molecular Medicine, Germany

*Massively Parallel Lineage Tracing Using CRISPR/Cas9*

**Birgit C. Schultes**, Intellia Therapeutics, USA

*Developing Next-Generation Engineered TCR-T Cells with CRISPR*

Short Talks Chosen from Abstracts

#### Next Generation Immune Cell Engineering (Q1)

**Crystal L. Mackall**, Stanford University, USA

*Engineering Enhanced Potency T Cells*

**Chiara Bonini**, Ospedale San Raffaele, Italy

*Multiple Genome Editing of Early Differentiated T Cells for Cancer Immunotherapy*

**Christopher A. Klebanoff**, Memorial Sloan Kettering Cancer Center, USA

*Targeting Solid Malignancies with "Public" Neoantigen TCRs*

**Aude G. Chapuis**, Fred Hutchinson Cancer Research Center, USA

*Enhancing the Efficacy of eTCR Therapies for Solid Tumors*

Short Talks Chosen from Abstracts

#### Workshop 2: Bacterial Adaptive Immunity Anti-CRISPR (Q2)

Short Talks Chosen from Abstracts

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### Workshop 2: HSC and iPSC Engineering and Therapy (Q1)

#### Short Talks Chosen from Abstracts

### Translational Science of Genome Editing: Assessing Specificity, Immunogenicity and Safety (Q2)

**J. Keith Joung**, Massachusetts General Hospital, USA  
*Advances in Identifying and Modulating the Specificities of Gene Editing Technologies*

**Zuben E. Sauna**, Food and Drug Administration, USA  
*Understanding and Navigating the Immune Responses to CRISPR-Associated Nuclease Cas9*

#### Speaker to be Announced

#### Short Talks Chosen from Abstracts

### Cell Therapy for Infection and Autoimmune Diseases (Q1)

**Jeffrey A. Bluestone**, University of California, San Francisco, USA  
*Manipulating Tregs to Control Tolerance in Autoimmunity and Organ Transplantation*

**Bruce R. Blazar**, University of Minnesota, USA  
*Treg Therapies in the Setting of Allogeneic HSCT*

**Hans-Peter Kiem**, Fred Hutchinson Cancer Research Center, USA  
*Engineering Resistance to HIV Infection*

**Megan K. Levings**, University of British Columbia, Canada  
*CAR-Treg-based Therapeutics*

#### Short Talks Chosen from Abstracts

### Poster Session 2

## TUESDAY, FEBRUARY 11

### CRISPR-Based Editing of Non Immune Cells (Joint)

**Matthew Porteus**, Stanford University School of Medicine, USA  
*CRISPR Editing of Human HSCs*

**Alex Marson**, University of California, San Francisco, USA  
*Reprogramming Human Immune Cell Circuitry*

**Jacob E. Corn**, ETH in Zurich, Switzerland  
*How Human Cells Deal with Cas-Based Tools*

**Scot A. Wolfe**, University of Massachusetts Medical School, USA  
*Precise Therapeutic Gene Correction by a Simple Nuclease-Induced Double-Strand Break*

#### Short Talks Chosen from Abstracts

### Workshop 3: Regulatory Science (Joint)

#### Short Talks Chosen from Abstracts

### “Off the Shelf” Allogeneic Cell Therapies (Joint)

**Daniel Shoemaker**, Fate Therapeutics, USA  
*Next Generation iPSC Derived Cell Therapies for Cancer*

**Hanspeter Waldner**, CRISPR Therapeutics, USA  
*Development of an Allogeneic T Cell Therapy for Oncology*

**Sonja Schrepfer**, University of California San Francisco, USA  
*Hypoimmunogenic Derivatives of Induced Pluripotent Stem Cells Evade Immune Rejection in Fully Immunocompetent Allogeneic Recipients*

#### Short Talks Chosen from Abstracts

### Poster Session 3

## WEDNESDAY, FEBRUARY 12

### DNA Repair and Genome Editing (Q2)

**James E. Haber**, Brandeis University, USA  
*Understanding Cas9-Mediated Gene Targeting by Single-Strand Template Repair*

**Vic E. Myer**, USA  
*DNA Repair and Genomic Stability in Primary Human Cells*

**Albert B. Seymour**, Homology Medicines, USA  
*AAVHSC-Mediated Genome Editing through Homologous Recombination: Applications in Liver-Mediated Diseases*

**Gaëlle Legube**, Center for Integrative Biology, France  
*Transcription Coupled DNA Double Strand Break Repair: Dealing with Breaks on Transcribed Loci*

#### Short Talks Chosen from Abstracts

### Using Synthetic Biology to Enhance Safety and Efficacy of Cellular Therapeutics (Q1)

**Yvonne Y. Chen**, University of California, Los Angeles, USA  
*Synthetic Biology to Engineer More Potent CAR T Cells*

**Kole T. Roybal**, University of California, San Francisco, USA  
*Engineering Next-Generation Immune Cell Therapies for Solid Tumors*

**Marius Wernig**, Stanford University, USA  
*iPSC Derived Cells Engineered with CRISPR for Treatment of Epidermolysis Bullosa*

#### Short Talks Chosen from Abstracts

### New Genome Editing Tools (Q2)

**David R. Liu**, Broad Institute, HHMI, and Harvard University, USA  
*Base Editing and Prime Editing: Genome Editing Without Double-Strand Breaks*

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**Erik Sontheimer**, University of Massachusetts Medical School, USA  
*Editing Genomes with Engineered Guide RNAs and Compact, Accurate Cas9 Homologs*

**Yan Zhang**, University of Michigan, USA  
*Long-Range Genome Engineering in Human Cells with CRISPR-Cas3*

**Short Talk Chosen from Abstracts**

### Non-T Immune Cell Therapies (Q1)

**Katy Rezvani**, University of Texas MD Anderson Cancer Center, USA  
*NK-CAR T Cells*

**Jeffrey S. Miller**, University of Minnesota, USA  
*Novel Strategies to Activate and Target NK Cells as Off-The-Shelf Therapy to Treat Cancer*

**Leonid S. Metelitsa**, Baylor College of Medicine, USA  
*NKT Cells as a Platform for Adoptive Cancer Immunotherapy*

**Short Talk Chosen from Abstracts**

**Meeting Wrap-Up: Outcomes and Future Directions (Organizers)**  
(Q2)

**Meeting Wrap-Up: Outcomes and Future Directions (Organizers)**  
(Q1)

**THURSDAY, FEBRUARY 13**

**Departure**