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Collide Pioneering Stem Cell Research With Precision CRISPR Genome Editing to Power Your Research From Basic Biology to Regenerative Medicine

Expert Speakers Include:

Ru Gunawardane
Director, Stem cells & Gene editing
Allen Institute

Bruce R. Conklin
Investigator
Gladstone Institutes & UCSF

George Church
Professor
Harvard & MIT

Jacob Corn
Principal Investigator
Berkeley

Jennifer Mitchell
Associate Professor
University of Toronto

Gene Yeo
Professor of Cellular & Molecular Medicine
UCSD

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Welcome to the inaugural CRISPR Stem Cell Congress 2018

The advancements of CRISPR technology and the progression in stem cell biology seems like a match made in heaven.

One that opens up the possibility to truly further the understanding of disease biology, to utilize the therapeutic and clinical applications of stem cells and to open up the door to the enhanced potential of regenerative medicine.

Join the inaugural **CRISPR Stem Cell Congress, in partnership with the Allen Institute for Cell Science,** to navigate the intricacies of, and overcome the current limitations of CRISPR editing in the maturing field of stem cell research. Learn how to intersect the two to mould our understanding of human genetics, developmental biology, and regenerative medicine.

The **CRISPR Stem Cell Congress** showcases cutting edge data from key opinion leaders who are pushing the boundaries of CRISPR in both basic biological research and translational clinical development of stem cell therapies. Dissect the challenges around optimizing guide RNA design, upping editing efficiency & utilizing novel delivery - Discover how to revolutionize human stem cell technologies, overcome the scientific difficulties of editing human stem cells and understand the boundless potential for regenerative medicine.

Hear what previous attendees have to say

"Thank you for having me. I have attended all CRISPR Congresses and they keep getting better"

John Sundman, Past attendee, Precision CRISPR Congress 2018

"I greatly enjoyed the diversity and topic coverage of the talks, thanks for putting together an excellent program!"

Christina Kolodzy, Syros Pharmaceuticals, Past attendee, Precision CRISPR Congress 2018

"Overall, I was very impressed again with this conference and the vast majority of topics covered"

Mike Peoples, MD Anderson, Past attendee, Precision CRISPR Congress 2018

5 Key Takeaways from the 2018 CRISPR Stem Cell Congress

1.

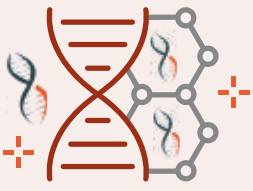
Learn how to utilize CRISPR/Cas9 to **illuminate stem cell organization and dynamics** to use a systematic approach to generate knock-ins in stem cells with insights from the **Allen Institute for Cell Science**

2.

Discover a **patient's journey** through regenerative stem cell treatment from patient advocate, **Douglas Oliver**

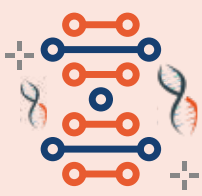
3.

Discover how **Gladstone Institutes** utilize CRISPR and Stem Cells to forward disease mechanism and genome surgery



4.

Understand how to re-program stem cells into virtually any set of cell types once the RNA profile of the cell targets are known with learnings from **George Church**



5.

Learn how **modulating RNA** diseases using CRISPR/Cas technology can be applied to diseases with **Gene Yeo, UCSD**

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hansonwade

Expert Speakers

Ru Gunawardane
Director, Stem cells and Gene Editing
Allen Institute for Cell Science

Rick Horwitz
Executive Director
Allen Institute for Cell Science

Bruce R. Conklin
Investigator
Gladstone Institutes & UCSF

George Church
Professor
Harvard & MIT

Beno Freedman
Assistant Professor
University of Washington

Fyodor Urnov
Associate Director
Altius Institute for Biomedical Sciences

Dirk Hockemeyer
Assistant Professor
Berkeley

Jennifer Mitchell
Associate Professor
University of Toronto

Gene Yeo
Professor of Cellular & Molecular Medicine
UCSD

Doug Oliver
Founder-Chair
Regenerative Medicine

Adriana Beltran
Director
UNC

Martin Kampmann
Assistant Professor
UCSF

Natasha Snider
Assistant Professor
UNC-Chapel Hill

Dane Hazelbaker
Research Scientist
Broad Institute

Kristin Baldwin
Professor
The Scripps Research Institute

Jacob Corn
Principal Investigator
Berkeley

William Hendriks
Instructor
Massachusetts General Hospital

Linzhao Cheng
Professor of Medicine & Oncology
John Hopkins Medical School

David Piper
Director, Research & Development
Thermo Fisher

Ania Wronski
Field Application Scientist
Synthego

Nelly Cruz
Research Scientist
University of Washington

Conference Day One | Wednesday, June 13th 2018

8.00 Registration

8.30 Chair's Opening Remarks

CRISPR as a Cure – the Therapeutic Success of CRISPR in Stem Cells & the Potential for More

Ru Gunawardane
Director, Stem cells & Gene editing
Allen Institute for Cell Science

8.40 Using CRISPR/Cas9 to Illuminate Stem Cell Organization & Dynamics

- A systematic approach for generating knock-ins in stem cells
- Identifying and improving precision of HDR events
- Use of live imaging and gene editing to explore cell states

Doug Oliver
Founder-Chair
Regenerative Medicine

9.00 When Science Needs The Power Of Story

- Account of self-directed research
- Raising funding and public/press response
- Pathway to recovery
- Patient advocacy tipped the scale for regenerative medicine funding

David Piper
Director, Research & Development
Thermo Fisher

9.30 Eliminating Inherent Genome Editing Bottlenecks in IPSCs to Build Physiologically Relevant Disease Models

- A review of strategies that leverage induced pluripotent stem cells (iPSCs) to increase the relevance of human cell models for in vitro approaches
- Discussion of current advances in genome engineering to pivot from challenges with delivery, identification and selection and subsequent clonal outgrowth by leveraging tools to consistently and reliably generate knock-out and knock-in models for use in target or compound identification
- We demonstrate this approach with iPSCs to build isogenic disease models, which can be further differentiated to various cell types of interest such as cardiomyocytes and dopaminergic neurons to model disease and are more directly related to a disease area than commonly used immortalized cell lines

10.00 Speed Networking

10.20 Morning Refreshments

Fyodor Urnov
Associate Director
Altius Institute for Biomedical Sciences

10.40 Genome Editing B.C.: Lasting Lessons From the "Old Testament"

- Genome editing with engineered nucleases, a powerful tool for understanding biological function and revealing causality, was built in a joint effort by academia and industry in 1994-2010
- Use of CRISPR-Cas9 is the most recent (2013-), and facile, implementation of the resulting editing toolbox
- Principles and methods of genome editing from the pre-CRISPR era remain relevant and continue to be useful

Bruce R. Conklin
Investigator
Gladstone Institutes & UCSF

11.10 CRISPR & Stem Cells: Disease Mechanism & Genome Surgery

- Choosing the right iPSC for the right experiment. The value of isogenic lines
- Building Genome Surgery as field, while developing treatments for specific diseases
- Dominant negative diseases of the retina and motor neurons are early genome surgery targets

Dirk Hockemeyer
Assistant Professor
Berkeley

11.40 Elucidating the Principles of Cancer Cell Immortality Using Genetically Defined Human Stem Cell Models

- Proliferation is required for long-term self-renewal and maintenance of human tissue, but must be limited to prevent uncontrolled growth associated with tumorigenesis
- TERT is expressed in stem cell compartments, but repressed in most somatic cell types. In order to understand the regulatory mechanisms of TERT expression in stem cell and upon differentiation, we study endogenous cis- and trans-elements of the human TERT promoter in human pluripotent stem cells using genome editing
- We have identified the elements that are required for the expression of TERT in the stem cell state

12.10 Networking lunch

13.15 Meet in the Municipal Room (Exhibit room) for Organized Travel to the Allen Institute

Afternoon Sessions Held at the Allen Institute: Back to Basics – Furthering Biological Understanding With the Use of Genetically Manipulated Stem Cells

Rick Horwitz
Executive Director
Allen Institute for Cell Science

14.00 Welcome Presentation from the Allen Institute

George Church
Professor
Harvard & MIT

14.30 Genome & Epigenome Engineering in Organs & Organoids

- Overcoming the special challenges of CRISPR HR in pluripotent stem cells, first published in Jan 2013, has now advanced to full nuclear transfer pig cloning in 2017 for xeno transplantation via apoptosis inhibitors
- We also have a potent path to re-programing stem cells into virtually any set of cell types once we know the RNA profile of the cell targets

Jacob Corn
Principal Investigator
Berkeley

15.00 Gene Repair in Human Hematopoietic Stem Cells

- Mechanisms of HDR in human cells
- Challenges of editing in hematopoietic stem cells
- Progress towards translation of gene editing therapies for sickle cell disease

15.30 Afternoon Refreshments

Gene Yeo
Professor of Cellular & Molecular Medicine
UCSD

16.00 Modulating RNA Diseases Using CRISPR/Cas Technology

- We are studying RNA diseases such as myotonic dystrophy, huntington's and ALS.
- RNA-targeting Cas proteins can modulate and destroy toxic RNA
- We will present in vivo use of these technologies

Kristin Baldwin
Professor
The Scripps Research Institute

16.30 Haplotype Editing IPSCs to Demystify Human-Specific & Non-Coding Genomic Disease Risk loci

- Human genetic studies frequently identify risk NSPs or haplotypes in non-coding and human-specific disease risk loci that are not well modeled in rodents
- The 9p21.3 cardiovascular disease risk region is primate specific, contains no coding genes and accounts for ~10-15% of coronary artery disease incidence in the US
- In IPSCs from risk and protected patients we deleted the entire ~60kb haplotype and performed RNA-Seq throughout differentiation of vascular smooth muscle cells – a strongly implicated causative cell type
- Deletion of the risk haplotype specifically rescues VSMC function and identifies new target molecules and pathways relevant to cardiovascular disease

17.00 Chair's Closing Remarks

17.15 Drinks & Poster Session Hosted by the Allen Institute for Cell Science

ALLEN INSTITUTE for CELL SCIENCE

Conference Day Two | Thursday, June 14th 2018

8.30 Morning Coffee & Refreshments

9.00 Chair's Opening Remarks

A Continued Discussion of Using CRISPR for Regulating the Genome

Jennifer Mitchell
Associate Professor
University of Toronto

9.10 Enhancer Switching Regulates Sox2 During the Transition From Embryonic to Neural Stem Cells

- CRISPR-mediated enhancer deletion shows the enhancers required to regulate Sox2 in embryonic and neural stem cells are different
- Chromatin architecture is altered co-incident with the switch in enhancer usage
- Transcription factors bind intergenic regions in ES cells that are poised to regulate Sox2 in neural stem cells

Martin Kampmann
Assistant Professor
UCSF

9.40 Elucidating Disease Mechanisms & Therapeutic Targets in Human IPSC Models by CRISPR-Based Functional Genomics

- We have established genetic screens based on CRISPR, CRISPR interference (CRISPRi) and CRISPR activation (CRISPRa) in human iPSC-derived cell types
- Genetic modifier screens in patient-derived iPSCs and isogenic CRISPR-corrected lines can reveal mechanisms of disease-associated genes and therapeutic strategies
- We have established cellular phenotypes relevant for neurodegenerative diseases in human iPSC-derived neurons, astrocytes and microglia and conducted first genetic modifier screens in a pooled format
- We are also establishing a platform for CRISPR-based arrayed high-content screens in iPSC-derived cells to investigate complex and non-cell autonomous phenotypes

Natasha Snider
Assistant Professor
UNC-Chapel Hill

10.10 Combining Patient-Derived IPSCs & CRISPR Technology in Disease Modeling & Drug Discovery for Rare Genetic Disorders

- "Cluster-and-conquer" approach for >80 rare human diseases linked to the intermediate filament gene family
- CRISPR/Cas9 gene editing of Alexander Disease and Giant Axonal Neuropathy patient iPSCs to obtain isogenic control cells
- 2D and 3D differentiation platforms using disease mutant and isogenic control iPSCs to generate astrocytes and neurons
- Validation of disease targets and testing of small molecule compounds in iPSCs-derived astrocytes

10.40 Morning Refreshments

Dane Hazelbaker
Research Scientist
Broad Institute

11.10 A Scaled Framework for CRISPR Editing of Human Pluripotent Stem Cells to Study Psychiatric Disease

- We report a standardized CRISPR-Cas9 approach with robust benchmarking at each step to edit a large set of psychiatric disease-implicated genes in hPSCs
- Transcriptional state and nucleosome positioning around targeted loci are not correlated with CRISPR editing efficiency
- Editing frequencies vary between different hPSC lines and correlate with genomic stability
- Precision editing with small and large ssDNAs provides an efficient platform for future editing frameworks

Ania Wronski
Field Application Scientist
Synthego

11.40 Synthetic sgRNA Enables Highly Efficient & Consistent CRISPR Editing of Cells for Automation, Cell Engineering & Therapeutic Applications

- Achieving consistent and high editing efficiencies with CRISPR is critical for automation, cell engineering and therapeutic applications with primary cells, and remains a significant challenge
- Through a collaborative effort, we demonstrate that use of synthetic sgRNA for CRISPR yields improved and consistent editing efficiencies that are required for such applications

Linzhao Cheng
Principal Investigator
John Hopkins Medicine

11.55 Precise Genome Editing in Human Stem Cells *Ex Vivo*

12.25 Lunch & Networking

Beno Freedman
Assistant Professor
University of Washington

13.25 Using CRISPR to Illuminate Organs and Organoids

- Organoids are multicellular units in vitro that resemble specific tissues or organs of the body (e.g. mini-brains and mini-intestines)
- Kidney organoids derived from Allen Institute iPS cell lines reveal organelle dynamics at the tissue scale
- CRISPR can be used to knock out whole organelles in human tissues
- Using CRISPR in embryos allows the growth of entire organs from a different species in mice

William Hendriks
Instructor
Massachusetts General Hospital

13.55 Human IPSC-Based Disease Modeling Reveals a Retrotransposon With Repeat Expansion as the Causal Mechanism of X-Linked Dystonia Parkinsonism

- Human iPSC-based XDP disease modeling reveals causative mutation of XDP
- An XDP-specific SVA insertion induces intron retention and down-regulation of TAF1
- CRISPR/Cas9 excision of SVA rescues aberrant splicing and cTAF1 expression in XDP
- Expression profiling implicates neurodevelopment and dystonia pathways in XDP iPSC-derived neural cells

Adriana Beltran
Director
UNC

14.25 CRISPRing iPSCs to Efficiently Model Genetic Diseases

- Generating iPSC from patients with genetic disorders
- Creating isogenic iPSCs for disease modeling with the CRISPR/Cas9 system
- Applying the CRISPR/Cas9 system to Cerebral Cavernous Malformation (CCM) patientspecific iPSCs to generate knockout models
- Developing 3D culture platforms of iPSCs-derived endothelial cells for transcriptome and proteomic studies

14.55 Afternoon Refreshments

15.25 Round table sessions

1. Discuss the Novel Therapeutic Applications of CRISPR Gene Editing in Stem Cells

2. Delve Into the Potential of Next Generation Disease Modeling & Drug Screening by Utilizing Genetically Engineered Stem Cells

3. Back to Basics – Where Does the Potential Lie for Manipulating Stem Cells in the Context of Basic Research?

16.25 Chair's Closing Remarks

16.30 Close of CRISPR Stem Cell Congress 2018

Pre-Conference Workshop Day | Tuesday 12th June

Workshop A

The Potential for CRISPR + Stem Cells to Help Patients Tame "The Wild West"

11:00am – 2:00pm

- Hope, Ambiguity, & the Imminence of "The Miracle Factor"
- The Need For Curated Information In The Public And Academic Communities
  - a) The Diagnosis Dilemma (Phenotype) and public / patient inferences
  - b) Redefinition Of "The Miracle Factor"
- Stem Cells 2.0—How CRISPR / Stem Cell Treatments Can Help Tame "The Wild West"

Workshop Leader
Doug Oliver
Founder-Chair
Regenerative Medicine

Doug is a writer, editorial contributor, and patient advocate from Nashville, Tennessee. He holds a Master's Degree in Social Work from the University of New England and has specialized in work on health care policy, compliance, and clinical case advocacy.

He is also a stem cell patient. At age 32, he was diagnosed with macular degeneration, a disease that attacks the retina, causing blindness of central vision. Doug became legally blind at age 45. In August 2015, he underwent autologous bone-marrow stem cell therapy to both eyes, and regained much of his lost vision. He received his driver's license 5 months later. Doug's story has reached millions through international broadcasts, interviews with the national press, and countless affiliate television and news media outlets.

Workshop B - Hosted by Thermo Fisher Scientific

Practical Insights, Consideration, & Discussion for Optimizing Genome Editing of Stem Cells

3:00pm – 6.00pm

This workshop will offer:

- Considerations and application techniques for successfully editing stem cells in your lab
- Review of stem cell workflows optimized for improved editing outcomes
- Panel discussion regarding common challenges, tips and tricks, and future direction of editing as applied to stem cell research. Come with your questions or discussion topics for panelists

Speakers Include:

Workshop Leader
Ru Gunawardane
Director, Stem cells & Gene Editing
Allen Institute for Cell Science

FOUNDING PARTNER

ALLEN INSTITUTE for CELL SCIENCE

Founded in 2003 by Paul G. Allen, the Allen Institute has expanded from its initial pursuit of understanding the brain to encompass an investigation of the inner workings of cells and the funding of transformative scientific ideas around the world.

The Allen Institute for Cell Science is a division of the Allen Institute and is dedicated to understanding and modeling cells: the fundamental units of life. By integrating technologies, approaches, models and data into a common standardized framework, the Allen Institute for Cell Science is creating dynamic, visual models of how genetic information is transformed into cellular behavior, and how the molecules and organelles within each cell interact and function as systems. These predictive models will enable the cell science community to better understand the role of cells in both health and disease. The Allen Institute for Cell Science's data and tools are publicly available online at:

cellscience.alleninstitute.org

The afternoon of day one will be held at the Allen Institute, a 270,000-square-foot life sciences building, at the northwest corner of Mercer St. and Westlake Avenue North in the South Lake Union neighborhood of Seattle. The building is purpose-built for life science research and facilitates our trademark team science approach amid abundant natural light and with stunning views of Seattle.The building design incorporates the historic elements of the Ford and Pacific McKay buildings, including the terra cotta facades that were located on the site previously and have been disassembled, restored and reinstalled. At six stories, the building also includes about 9,000 square feet of ground floor retail space and two levels of subterranean parking.

Find out more: cellscience.alleninstitute.org

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Synthego is a leading provider of genome engineering solutions. The company's flagship product, CRISPRevolution, is a portfolio of synthetic RNA designed for CRISPR genome editing and research. Synthego's vision is to bring precision and automation to genome engineering, enabling rapid and cost-effective research with consistent results for every scientist. Headquartered in Silicon Valley, California, Synthego customers include leading institutions in 31 countries around the world, and 9 of the top 10 global biology universities.

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Aldevron serves the biotechnology industry with custom production of nucleic acids, proteins, and antibodies. Thousands of clients use Aldevron-produced plasmids, RNA and gene editing enzymes for programs ranging from research to clinical trials to commercial applications. Aldevron's Cas9 nuclease is available as an off-the-shelf product at research grade, GMP-Source and GMP quality levels, eliminating months typically associated with contract production.

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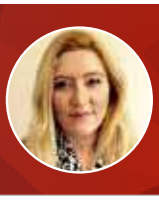

Diane McKenna
Commercial Director, CRISPR Series
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TOP 3 BENEFITS OF ATTENDING:

1. Engage in debate to dissect the challenges around optimizing guide RNA design, upping editing efficiency & utilizing novel delivery methods to boost efficiency of gene editing stem cells
2. Discover the next generation of CRISPR editing in stem cells to harness the therapeutic and clinical potential
3. Delve into the potential of next generation disease modeling and drug screening by utilizing genetically engineered stem cells

Team Discounts*

15% - 3 delegates
20% - 4 delegates
25% - 6 or more delegates

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SECURE YOUR PLACE

Industry Pricing	Register before Friday April 20	Standard Pricing
Conference + 2 Workshops	$2,797 **(Save $600)**	$2,997 **(Save $400)**
Conference + 1 Workshop	$2,298 **(Save $400)**	$2,498 **(Save $200)**
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https://www.marriott.co.uk/hotels/hotel-photos/seasm-renaissance-seattle-hotel/

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