Genome Engineering – Cutting-Edge Research and Applications
June 22 – 27, 2014
Nassau, Bahamas

Co-Organizers:

J. Keith Joung
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Boston, MA, U.S.A.

Thierry Vandendriessche
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Brussels, Belgium

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La Jolla, CA, U.S.A.

Sunday, June 22, 2014	
4:00PM – 9:00PM	Conference Registration
6:00PM – 7:00 PM	FASEB Opening Reception
7:00PM – 8:30 PM	Dinner
8:30PM - 8:45PM	Opening remarks
8:45PM – 10:00PM	**Keynote Speaker**: Luigi Naldini, Telethon Institute of Gene Therapy - San Rafaelle Institute, Milano *"Targeted genome editing in human repopulating hematopoietic stem cells"*
Monday, June 23, 2014	
7:30AM – 9:00AM	Breakfast

Session 1: **9:00AM – 12:15PM**	**DNA damage response: mechanisms and applications** Session Chair: Maria Jasin, Memorial Sloan-Kettering, New York
9:00AM – 9:15AM	Welcome from FASEB
9:15AM - 9:45AM	Nancy Maizels, University of Washington, Seattle *"Distinct pathways support gene correction at nicks and double-strand breaks"*
9:45AM –10:15AM	Maria Jasin, Memorial Sloan-Kettering, New York *"Homologous recombination repair of double-strand breaks and nicks"*
10:15AM – 10:45 AM	Group photo & FASEB Sponsored Coffee Break
10:45AM – 11:15AM	Lee Zou, Massachusetts General Hospital, Boston *"Regulation of DNA damage signaling by protein SUMOylation"*
11:15AM – 11:30AM	Eric Hendrickson, University of Minnesota, Minneapolis *"The mechanism of gene targeting in human somatic cells" (Poster A29)*
11:30AM – 11:45AM	Marcello Maresca, AstraZeneca, Molndal, Sweden *"ObLiGaRe: targeted integration by homology independent repair in cells and animals" (Poster B3)*
Session 2: **11:45AM – 12:15PM**	**Engineering customized nucleases (I)** Session Chair: Greg Davis, Sigma-Aldrich Biotechnology, St. Louis
11:45AM – 12:15PM	Greg Davis, Sigma-Aldrich Biotechnology, St. Louis *"Genome editing with group II introns, ZFNs, CRISPR, and alternative donor DNA formats"*
session continues in the evening…	
12:15PM – 1:15PM	Lunch & **Meet-the-Investigator** (Keynote Speaker and speakers from Session 1 and 2)
	Free Afternoon
3:30PM – 5:30PM	**Poster Session 1 (Poster group A)**
5:30PM – 7:30PM	Dinner
Session 2: **7:30PM – 10:00PM**	**Engineering customized nucleases (I) (continued)** Session Chair: Greg Davis, Sigma-Aldrich Biotechnology, St. Louis
7:30PM – 7:45PM	Alexander Hruscha, Ludwig-Maximilians University, Munich *"Improved CRISPR/Cas9 toolbox for zebrafish" (Poster A31)*
7:45PM – 8:00PM	Rama Mangena, Horizon Discovery Plc, Cambridge, UK *"Improved gene editing efficiencies using AAV donors in combination with nuclease based approaches" (Poster B2)*

8:00PM – 8:30PM	Barry Stoddard, Fred Hutchinson Cancer Research Center, Seattle *"Genome engineering in* Anopheles Gambiae*: combatting malaria with engineered meganucleases"*
8:30PM – 8:45PM	Shengdar Tsai (##), Massachusetts General Hospital, Boston *"A dimeric next-generation CRISPR-based nuclease platform for efficient genome editing" (Poster B29)*
8:45PM – 9:15PM	Edward J. Rebar, Sangamo BioSciences, Inc., Richmond *"Design and optimization of sequence-specific nucleases for genome engineering"*
9:15PM – 9:45PM	Jens Boch, Martin-Luther University Halle-Wittenberg, Halle *"A flexible TALE DNA-binding domain"*
9:45PM—10:00PM	Tetsushi Sakuma, Hiroshima University, Japan *"Genome editing using Platinum TALENs" (Poster B19)*
Tuesday, June 24, 2014	
7:30AM – 9:00AM	Breakfast
Session 3 9:00AM – 12:00PM	**Engineering customized nucleases (II)** Session Chair: J. Keith Joung, Massachusetts General Hospital, Boston
9:00AM - 9:30AM	J. Keith Joung, Massachusetts General Hospital, Boston *"Targeted genome & epigenome editing using engineered CRISPR and TALE technologies"*
9:30AM – 9:45AM	Mazhar Adli, University of Virginia, Charlottesville *"Monitoring genome level off-targets of CRISPR system reveals sequence determinants of Cas9 binding specificity" (Poster A2)*
9:45AM – 10:00AM	Gang Bao, Georgia Institute of Technology, Atlanta *"CRISPR/Cas9 systems have off-target activity with insertions or deletions between target DNA and guide RNA sequences" (Poster A7)*
10:00AM – 10:15AM	Rachel Haurwitz, Caribou Biosciences, Inc., Berkeley, CA *"Distinct guide RNA features direct Cas9 activity" (Poster A28)*
10:15AM – 10:45AM	FASEB Sponsored Coffee Break
10:45AM – 11:15AM	Jin-Soo Kim, Seoul National University, Seoul *"Genome engineering in cultured cells and whole organisms with programmable nucleases"*
11:15AM – 11:45AM	Emmanuelle Charpentier, Helmholtz Centre for Infection Research *"Functional evolution of RNA-programmable Cas9 for an improved genome editing toolbox"*
11:45AM – 12:00PM	David Edgell, Western University, London, Ontario *"Genome-editing applications of the GIY-YIG nuclease domain:*

	monomeric TALENs and dual-active site MegaTevs" (Poster A22)
12:15PM – 1:15PM	Lunch & **Meet-the-Investigator** (Speakers from Sessions 3 & 4)
	Free Afternoon
5:30PM – 7:30PM	Dinner
Session 4: **7:30PM – 10:00PM**	**Emerging principles of synthetic biology for genome engineering** Session Chair: James Collins, Boston University, Boston
7:30PM – 8:00PM	James Collins, Boston University, Boston *"Synthetic biology: biomedical applications come of age"*
8:00PM – 8:30PM	Christina Smolke, California Institute of Technology, Pasadena *"Designing synthetic regulatory RNAs: new tools for temporal and spatial control in biological systems"*
8:30PM – 8:45PM	Tadas Jakociunas (##), Technical University of Denmark *"Multiplexed genome engineering for terpenoid production in yeast" (Poster A33)*
8:45PM – 9:15PM	Miki Imanishi, Kyoto University, Kyoto *"Manipulation of the cellular clock using artificial transcription factors"*
9:15PM – 9:45PM	Thierry VandenDriessche, Free University, Brussels "*Genome-wide* in silico *identification of* cis*-regulatory transcriptional modules conferring high tissue-specific gene expression: an emerging platform for gene therapy and genome engineering*"
9:45PM – 10:00PM	Lior Nissim (##), Massachusetts Institute of Technology, Cambridge, MA *"An integrated RNA and CRISPR/Cas toolkit for multiplexed synthetic circuits and endogenous gene regulation in human cells" (Poster B10)*
Wednesday, June 25, 2014	
7:30AM – 9:00AM	Breakfast
Session 5 **9:00AM – 11:30AM**	**Technologies for controlling gene expression** Session Chair: Gerd Blobel, Children's Hospital of Pennsylvania, Philadelphia
9:00AM - 9:30AM	Le Cong, Broad Institute, Cambridge *"Genome engineering: technologies and applications"*
9:30AM –10:00AM	Pilar Blancafort, University of Western Australia, Perth *"Engineering the cancer epigenome: novel strategies to target breast and ovarian tumors"*

10:00AM – 10:15AM	Stephanie Byrum (##), University of Arkansas, Little Rock *"Purification of a specific native genomic locus for proteomic analysis" (Poster A12)*
10:15AM – 10:45AM	Gerd Blobel, Children's Hospital of Pennsylvania, Philadelphia *"Reprogramming the β-globin locus by forced chromatin looping"*
10:45AM – 11:15AM	Osamu Nureki, The University of Tokyo *"Crystal structure of Cas9 complexed with guide RNA and target DNA"*
11:15AM – 11:30AM	Pratiksha Thakore (##), Duke University, Durham, NC *"CRISPR/Cas9-based transcriptional repressors for eukaryotic gene regulation" (Poster B27)*
12:15PM – 1:15PM	Lunch & **Meet-the-Investigator** (Speakers from Sessions 5 & 7)
	Free Afternoon
5:00PM – 7:00PM	**Poster Session 2 (Poster Group B)**
7:00PM – 8:30PM	Dinner
Session 6: **8:30PM – 10:00PM**	**A collection of selected short talk abstracts** Session Chair: Thierry VandenDriessche, Free University, Brussels
8:30PM – 8:45PM	Benjamin Matthews (##), Rockefeller University, New York, NY *"Genome engineering with CRISPR/Cas9 in the mosquito* Aedes aegypti*" (Poster B5)*
8:45PM – 9:00PM	Bettina Schmid, Ludwig-Maximilians University, Munich, Germany *"CRISPR-based transcriptional regulation in zebrafish" (Poster B20)*
9:00PM – 9:15PM	Stefano Stella (##), University of Copenhagen, Denmark *"BuD, a helix-loop-helix DNA-binding domain for genome modification" (Poster B23)*
9:15PM – 9:30PM	Patrick Blackburn (##), Mayo Clinic, Rochester, MN *"Utilizing TALENs and a Sleeping Beauty transposon system to model a novel mutation in FAH identified in a family with early onset cirrhosis and hepatocellular carcinoma" (Poster A9)*
9:30PM – 9:45PM	Keiichiro Suzuki (##), Salk Institute for Biological Studies, La Jolla, CA *"Analysis of whole-genome mutational load in gene corrected human disease-specific iPSCs and enhancement of gene targeting by a TALEN-HDAdV hybrid vector" (Poster B25)*
9:45PM – 10:00PM	Open slot for late-breaking short talk abstract
Thursday, **June 26, 2014**	

7:30AM – 9:00AM	Breakfast
Session 7 **9:00AM – 12:00PM**	**Targeted genome modification with recombinases, integrases and transposases** Session Chair: Laurence Cooper, MD Anderson Cancer Center, Houston
9:00AM – 9:30AM	Thomas Gaj, The Scripps Research Institute, La Jolla *"Genome engineering with designer recombinases"*
9:30AM – 9:45AM	Ben Davies, Wellcome Trust Centre for Human Genetics, Oxford, UK *"Reprogramming meiotic recombination in the mouse" (Poster A19)*
9:45AM - 10:00AM	Perry Hackett, University of Minnesota, Minneapolis, MN *"Is non-viral gene therapy by liver-directed hydrodynamic delivery of Sleeping Beauty transposons feasible in large animals?" (Poster A27)*
10:00AM –10:30AM	Zuzsanna Izsvak, Max Delbruck Center, Berlin *"Genome engineering by transposable elements"*
10:30AM – 10:45AM	Lauren Polstein (##), Duke University, Durham, NC *"Spatiotemporal control of mammalian gene regulation using a light-inducible recombinase" (Poster B16)*
10:45AM – 11:15AM	FASEB Sponsored Coffee Break
11:15AM – 11:30AM	Brian Lamb (##), The Scripps Research Institute, La Jolla, CA *"Site-specific recombinases as a useful tool for evolving DNA binding proteins" (Poster B1)*
11:30AM – 12:00PM	Laurence Cooper, MD Anderson Cancer Center, Houston *"Nonviral gene transfer to genetically modify and genetically edit clinical-grade T cells"*
12:00PM – 12:30PM	Business Meeting
12:45PM – 1:45PM	Lunch & **Meet-the-Investigator** (Speakers from Sessions 8 & 9)
	Free Afternoon
6:00PM – 7:30PM	Dinner
Session 8: **7:30PM – 10:00PM**	**Genome engineering in model and non-model organisms** Session Chair: Barbara Meyer, University of California, Berkeley
7:30PM – 8:00PM	Barbara Meyer, University of California, Berkeley *"Genome editing across diverse nematode species"*
8:00PM – 8:30PM	Bo Zhang, Peking University, Beijing *"Genome manipulation with engineered endonucleases in zebrafish"*

8:30PM – 8:45PM	Stephanie Bannister (##), The University of Vienna, Austria *"The new worm on the block: using TALENs to investigate specific biological phenomena in non-conventional model organisms" (Poster A6)*
8:45PM – 9:15PM	Daniel Carlson, Recombinetics, Minneapolis *"Editing the livestock genome one basepair at a time"*
9:15PM – 9:45PM	Daniel Voytas, University of Minnesota, St. Paul *"Precise engineering of plant genomes"*
9:45PM – 10:00PM	Mark Cigan, DuPont Pioneer, Johnston, IA *"Targeted gene modifications, gene editing and trait integration in crop plants" (Poster A16)*
Friday, June 27, 2014	
7:30AM – 8:30AM	Breakfast
Session 9 8:30AM – 11:30PM	**Clinical translation of genome engineering** Session Chair: Michael Holmes, Sangamo Biosciences, Inc., Richmond
8:30AM - 9:00AM	Michael Holmes, Sangamo Biosciences, Inc., Richmond *"Genome editing with zinc finger nucleases"*
9:00AM – 9:30AM	Paula Cannon, University of Southern California *"Maximizing targeted gene editing in hematopoietic stem cells"*
9:30AM – 9:45AM	Eric Poeschla, Mayo Clinic, Rochester, MN *"TALEN Knockout of the HIV-1 integration cofactor LEDGF/p75" (Poster B15)*
9:45AM – 10:00AM	Break
10:00AM – 10:30AM	Charles Gersbach, Duke University, Durham *"Custom redesign of the genome for gene therapy and regenerative medicine"*
10:30AM – 10:45AM	Yongming Wang, Stanford University, Stanford, CA *"Genome editing of isogenic human induced pluripotent stem cells recapitulates long QT phenotype for drug testing" (Poster B32)*
10:45AM – 11:15AM	Toni Cathomen, Universitätsklinikum Freiburg *"Gene editing in pluripotent stem cells to model primary immunodeficiencies"*
11:15AM – 11:30AM	Closing Comments
11:30AM	Departures Boxed Lunches Available

Recipient of Young Investigator Travel Award

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