Genome Editing & Engineering Conference

Choosing the right gene editing technology

February 18-19, 2016, San Diego-CA, USA

The Genome Editing & Engineering Congress brings together the key industry leaders and researchers to address the concepts, challenges and state of art methods & applications of the genome editing tools like CRISPR/Cas9, TALENs, ZNFs & AAVs etc. The case study & sessions will reveal the potential application of Genome editing tools from the modern biomedical & therapeutic application .Special emphasis on CRISPR system addressing the concept, technology, challenges like off-target effects, efficiency improvement and delivery systems etc.

Attendees of the event will learn about:

- The cutting edge therapeutic application of Genome Editing tools: CRISPR/Cas9, TALEN, ZFN & AAVs
- How to overcome the challenges of CRISPR & other genome editing tools
- Advancements, challenges and future opportunities of CRISPR/Cas9 & other genome editing technologies
- The genome editing approaches to accelerate drug discovery, target identification, validation & screening
- The Genome Editing towards cell line engineering & disease model development
- Genome Editing application towards research animal models or transgenic animal
- The Regulatory challanges, Ethics and Intellectual property rights of Genome Editing technology

Conference Highlights

- 15+ case studies on Genome editing application
- Keynote by renowned experts
- Presentations from the pharma industry
- Open forum to discuss the best tool for your research

Confirmed Speakers:

Prof. Eric B. Kmiec
Director, Gene Editing Institute
Helen F. Graham Cancer Center & Research Institute
USA

Dr. Tod Woolf
Founder and President
ETAGEN Pharma
USA

Dr. Matthew Porteus
Principal Investigator
Stanford School of Medicine
USA

Prof. Radislav Sedlacek
Director, Czech Centre for Phenogenomics (BIOCEV/IMG)
Czech Republic

Dr. Dirk Hockemeyer
Principal Investigator
University of California Berkeley - USA

Dr. Rob Howes
Associate Director, HTS, Antibody Discovery and Protein Engineering
Medimmune
UK

Dr. Eric Paul Bennett
Associate Professor
Copenhagen Center for Glycomics (CCG)
Denmark

Prof. Fredy Altpeter
University of Florida - IFAS
USA

Dr. Raman Sood
Director, Zebrafish Core facility
NGRI-NIH
USA

Aimee B. Stablewski
Co-Director, Gene Targeting and Transgenic Resource
Roswell Park Cancer Institute, USA

Dr. Guangbin Xia
Department of Neurology
College of Medicine
University of Florida
USA

Dr. C. B. Gurumurthy
Director, Mouse Genome Engineering Core Facility,
University of Nebraska Medical Center, USA

Dr. Matthias Heidenreich
Post-Doctoral Fellow,
Feng Zhang Lab
Broad Institute, MIT
USA

Dr. Miguel A. Moreno-Mateos
Associate Research Scientist
Yale University School of Medicine, USA

Dr. Hao Yin
Research Scientist
David H. Koch Institute for Integrative Cancer Research, MIT, USA

Dr. Gaurav K. Varshney
National Human Genome Research Institute- NIH
USA

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MNM CONFERENCES

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February 18-19, 2016, San Diego-CA, USA

Day 1, Thursday, February 18th

08:00 **Registration & Refreshment**

08:50 **Opening remarks from the Chair**

09:00 Keynote Presentation

An Historical Perspective on the Development and Principles of Gene Editing Technologies

- Historical context, Comparision between Gene Therapy & genome Engineering, nomenclature, different mechanisms, methods and applications
- Classes of Edits or Modifications: Knockout, Knockin (gene or gene segments), Point Edits, Large Deletions

Prof. Eric B. Kmiec
Director, Gene Editing Institute Center for Translational Cancer Research
Helen F. Graham Cancer Center & Research Institute, USA

9:25 **Understanding the role of CRISPR/ Cas 9 in Genome Editing from technology overview to the future prospective**

- What are the advantages of CRISPR over other methods
- How to overcome the off-target Mutations
- What is the future of CRISPR/Cas9 system

Methods of Genome Editing and Engineering: Concept, Technology & Challenges

9:50 **CRISPR-revolution led paradigm-shifts in animal genome editing approaches**

- Introduction to the long-used traditional animal transgenic technologies, using mouse as a model organism.
- Paradigm shifts in animal transgenic technologies caused by the CRISPR/Cas9 system
- Latest advances in CRISPR/Cas9 genome editing platforms that have completely relieved the bottlenecks of long-used transgenic technologies

Dr. C. B. Gurumurthy
Director, Mouse Genome Engineering Core Facility, University of Nebraska Medical Center, USA

10:15 **Solution Provider Presentation**
Contact Steve Hambrook at steve.h@mnmconferences.com

10:45 **Morning Refreshments & Poster Presentations**

11:25 **Intro to Genome Editing and Engineering**

- RNA editing mechanisms and methods
- Applications of RNA editing
- New editing approaches in the context of prior editing literature
- Comparing therapeutic editing to gene therapy

Dr. Tod Woolf
Founder and President, ETAGEN Pharma, USA

11:50 **RNA guided genome engineering: new expansion of Cas9 toolbox and in vivo application**

- New animal models using CRISPR-Cas9
- In vitro genome editing in postmitotic neurons using SpCas9
- In vivo genome editing in the mouse brain using SpCas9
- Applications of SaCas9 for genome editing in brain and liver

Dr. Matthias Heidenreich
Post-Doctoral Fellow, Feng Zhang Lab, Broad Institute, MIT, USA

12:15 **Comparison of TALEN and RNAi for Targeted Mutagenesis or Target Gene Suppression in a Complex Genome**

- Vector construction
- In vitro gene delivery
- Alternative screening protocols to identify events with target gene knock-out and validation by sequencing
- Correlation of RNAi mediated target gene suppression with phenotype
- Rapid phenotyping protocol for knockout and knockdown events
- Comparison of performance of knockout or knockdown events

Prof. Fredy Altpeter
University of Florida-IFAS, USA

12:40 **CRISPRscan: Designing highly efficient sgRNAs for CRISPR/Cas9 targeting in vivo**

- A sgRNA-scoring algorithm capturing the sequence features affecting Cas9/sgRNA activity in vivo.
- Designing efficient alternative sgRNAs to increase the target site repertoire in the genome.
- Localizing Cas9 expression in the germ cells to reduce lethality and deleterious phenotypes in somatic tissues.

Dr. Miguel A. Moreno-Mateos
Associate Research Scientist
Department of Genetics, Yale University School of Medicine, USA

13:05 **Lunch Break and Poster Presentation | One-to-One Networking Meetings**

14:05 **Indel Detection by Amplicon Analysis (IDAA): A Novel and Improved Methodology for Genome Editing Surveillance**

- A novel Indel Detection by Amplicon Analysis (IDAA) method for genome editing applications.
- IDAA is based on a simple amplicon labelling strategy and automated Capillary Electrophoresis.
- IDAA is emnable to high throughput detection and characterization of indels induced by precise gene targeting.
- IDDA is cost effective and generates indel profiles similar to Sanger and "Deep Sequencing" with sub-percentage indel detection sensitivity.
- IDAA is highly useful for genome editing surveillance

Dr. Eric Paul Bennett
Associate Professor, Copenhagen Center for Glycomics (CCG), Denmark

Application of CRISPR & other Genome Editing Technologies: Cell Line Engineering, Therapeutic Application, Animal Model, Drug discovery & Screening

Genome Editing for Cell Line Engineering: Application of CRISPR/Cas9 & other tools

14:30 **Panel Discussion: CRISPR, ZFN, TALENS; Which technologies is the Best?**

14:55 **Explore the Genome editing application in mammalian cell line engineering**

- Application of mammalian cell line using CRISPR & expression of CRISPR Cas9 nuclease
- Efficient strategies for TALEN-based genome editing
- Integration of human AAVS1
- Knockout generation

15:20 **Disease modeling in Zebrafish using Genome Editing Tools**

- Efficient protocols for Generation of zebrafish knockout mutants for genes involved in human genetic diseases using ZFNs, TALENs and CRISPR/Cas9
- Comparison of ZFNS, TALENs and CRISPR/CAS9 mediated mutagenesis efficiency and types of mutations
- Phenotype of knockout mutants
- Status of targeted knock-in mutagenesis in zebrafish

Dr. Raman Sood,
Director, Zebrafish Core facility, National Human Genome Research Institute, National Institutes of Health, USA

15:45 **Afternoon Refreshment and Poster Presentation | One-to-One Networking Meetings**

16:30 **Application of Cell line engineering for Ex vivo Therapeutics**

- Cell line based therapeutics: Case study
- Cell-based therapies, protein drugs, gene therapies & vaccine.

16:55 **Understanding the regulatory Issues of Genome Editing**

- Why regulation is necessary?
- International regulations for ZFN and TALEN application
- Is there any regulation of CRISPR/Cas application?
- Genome editing is under GMO regulation or not?
- Regulatory approaches for Therapeutic application of CRISPR

17:20 **Chairman's Closing Remarks & Conference Close**

17:30 **Drinks Reception**

Genome Editing & Engineering Conference

Day 2, Friday, February 19th

8:00 **Registration & Refreshment**

8:20 **Opening remarks from the Chair**

8:30 Keynote Presentation:

Title: TBA

Dr. Matthew Porteus, Principal Investigator, Stanford School Of Medicine, USA

8:55 **Solution provider's presentation:** Keynote-2

Therapeutic Application of Genome Editing (CRISPR, ZFN, & TALEN)

9:25 **Elucidating Telomere Function in Human Tumor Biology**

- Mutations in the human telomerase reverse transcriptase (TERT) promoter are the most frequent non-coding mutations in cancer.
- We used genome editing to engineer these mutations in pluripotent stem cells.
- Telomerase-expressing embryonic stem with the cancer associated TERT promoter mutations showed only a modest increase in TERT transcription with no impact on telomerase activity.
- However, upon differentiation into somatic cells, which normally silence telomerase, cells with TERT promoter mutations failed to silence TERT expression, resulting in increased telomerase activity and aberrantly long telomeres.
- We conclude from these studies that TERT promoter mutations are sufficient to overcome the proliferative barrier imposed by telomere shortening without additional tumor-selected mutations.

Dr. Dirk Hockemeyer

Principal Investigator, University Of California, Berkeley - USA

9:50 **Morning Refreshments & Poster Presentations**

10:35 **Therapeutic in vivo delivery of CRISPR/Cas9 for next generation gene therapy**

- Development and characterization of RNA therapy delivery systems
- RNA delivery in: in in vivo & in vitro systems
- Discuss the successful RNA therapy in case of disease like-Viral infection, Hemophilia & other genetic disease

Dr. Hao Yin

Research Scientist, David H. Koch Institute for Integrative Cancer Research, MIT, USA

11:00 **Genome therapy for nucleotide repeat expansion-mediated neurodegenerative diseases**

- Introduction of monogenic, neurodegenerative diseases caused by nucleotide repeat expansion: Muscular dystrophies, Spinocerebellar Ataxias, Motor neuron disease.
- Mechanism of RNA/protein gain-of-function from nucleotide repeat expansion.
- Strategies of genome manipulation for nucleotide repeat expansion diseases
- Genome therapy of Myotonic Dystrophy Type 1 iPS cells
- Prospect of in vivo genome therapy.

Dr. Guangbin Xia

Department of Neurology, College of Medicine, University of Florida, USA

11:25 **Exploring Genome Editing Platform for immunotherapy**

- Engineering of human T Cells for therapeutic approach
- MegaTAL nucleases for stem cell and T cell therapies
- TALEN application for CAR-TCell generation
- Gene modification in T cells using editing tools

11:55 **High-throughput gene targeting using CRISPR/Cas9 for human disease modeling in zebrafish**

- High-throughput method of CRISPR/Cas9 gene editing in zebrafish
- Multiplex gene editing method
- Optimize sgRNA design for improved targeted efficiency
- Orthogonal Cas9 for expanding gene targeting coverage
- High-throughput phenotyping for studying function of human deafness genes in zebrafish.

Dr. Gaurav K. Varshney
National Human Genome Research Institute- NIH, USA

Genome Editing application for Drug Discovery & Screening: Use of CRISPR/Cas9 & other tools

12:20 **Explore the use CRISPR/Cas9 for high throughput screening (HTS)**

- How CRISPR/Cas9 is replacing existing technologies (e.g. RNAi)
- What are the advantages, limitations and challenges?
- What are the new types of screens that CRISPR/Cas9 enables (epigenetics, in-vivo, etc.)

Dr. Rob Howes
Associate Director, HTS, Antibody Discovery and Protein Engineering, MedImmune, UK

12:45 **Lunch and Poster Presentation | One-to-One Networking Meetings**

13:45 **Application of Gene Editing Technologies in Drug Discovery**

- How CRISPR fastens the early drug discovery process
- How Isogenic or gene-edited cell lines are used to identify the novel biologics

14:15 **Functional Screening with Lentiviral shRNA and CRISPR Libraries**

- Role of CRISPR/Cas9 Genome-Wide sgRNA Library Screening
- Genome-wide loss-of-function screening to identify regulatory genes
- Therapeutic target identification

Genome Editing application in Model organism

14:40 **Discussing the Genome editing application towards animal models**

- How to create Knockout, Transgenic, and Humanized Mice/ Rats/ Rabbits
- Understand the role of Zinc Finger Nuclease or CRISPR/Cas9 systems

Prof. Radislav Sedláček
Director, Czech Centre for Phenogenomics (BIOCEV/IMG), Czech Republic

15:05 **CRISPR mediated Genome editing in mice**

- Discuss the CRISPR/Cas9 technology platform to build mice models
- Safety assessment of therapeutics in animal model

15:30 **Afternoon Refreshment and Poster Presentation | One-to-One Networking Meetings**

16:15 **How Genome-Editing has Made the Generation of Genetically-Modified Animals Easier, and yet as Complicated as Ever**

- Brief history of genetically modifying animals via Transgenics, Knockouts/Knockins in ES cells, Lentiviral transgenics, SCNT, spermatogonial stem cell transfer and the advantages and disadvantages of each
- Genome editing technologies: ZFN, TALENs and CRISPR evolution with GEMM models and their impact on generation of species which previously could not be modified (large animal transgenics for research and agricultural production)
- Design, validation and purification of CRISPR guides for your GEMM project
- Quality control for your CRISPR injection project and Preparation of CRISPR reagents
- Analysis of the "founder" F0 CRISPR generation methods

Aimee B. Stablewski

Co-Director, Gene Targeting And Transgenic Resource, Roswell Park Cancer Institute, USA

16:40 **Ethics & Intellectual property rights (IPR) in Genome Editing**

- Ethical concerns and controversies of Genome Editing technologies
- Argument towards patent of CRISPR-Cas9
- International laws towards IPR in Genome engineering patents

17:05 **Chair person's closing remarks & Conference close**

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