Genome Editing & Engineering Conference

Choosing the right gene editing technology

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

The Genome Editing & Engineering Conference 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. Case studies and sessions will reveal the potential application of genome editing tools from the modern biomedical & therapeutic applications. Special emphasis on CRISPR system addressing the concept, technology, challenges like off-target effects, efficiency improvement and delivery systems.

Attendees at the event will learn about:

- Cutting edge therapeutic applications of genome editing tools: CRISPR/Cas9, TALEN, and ZFN
- Overcoming challenges in CRISPR and other genome editing tools
- Future opportunities of CRISPR/Cas9 and other genome editing technologies
- Genome editing approaches to accelerate drug discovery, target identification, validation and screening
- Genome editing towards cell line engineering and disease model development
- Genome editing application towards animal modeling or transgenic animal
- Regulatory challenges faced in genome editing

Conference Highlights

- 15+ case studies on genome editing applications
- Hands on workshop: Sequencing animal models and overcoming delivery system through CRISPR technology
- Presentations from the pharma industry
- Open forum to discuss the best tool for your research

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Confirmed Speakers:

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

Dr. Philippe Duchateau
Chief Scientific Officer,
Cellectis, France

Dr. Tod Woolf
Founder and President
ETAGEN Pharma
USA

Dr. John Feder
Associate Director of Genome Biology,
Bristol-Myers Squibb,
USA

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

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

Dr. Dirk Hockemeyer
Principal Investigator
University of California Berkeley
USA

Dr. Myung Shin
Associate Director
Merck, USA

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

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

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

Dr. Eric A Hendrickson
Professor,
Harvard Medical School,
Boston, MA, 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. C. B. Gurumurthy
Director, Mouse Genome Engineering Core Facility
University of Nebraska Medical Center, USA

Dr. Sandra Engle
Associate Research
Pfizer

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

Day 1, Thursday, February 18th

08:15 am **Registration & Refreshment**

08:45 am **Welcome Note from MnM Conferences**

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

09:00 am 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, DE, USA**

09:30 am **Hit and seek: Improved targeting and indel identification methodologies for genome editing**

- Gene editing workflow based on nuclease GFP tagging and a novel Indel Detection by Amplicon Analysis (IDAA) method
- IDAA is based on a simple amplicon labelling strategy and automated Capillary Electrophoresis.
- IDAA is enable 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**

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

10:00 am **Presentation:TBA**

Dr. Gregory Alberts, Field Sales and Service Manager, **Lonza Pharma Bioscience Solutions**

Lonza

10:30am **Morning Refreshments and Poster Presentation | One-to-One Networking Meetings**

11:10am **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, NE, USA**

11:40 am **Introduction 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, MA, USA**

12:10pm **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, MA, USA**

12:40pm **CRISPR scan: 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,CT, USA**

01:10pm **Lunch Break and Poster Presentation | One-to-One Networking Meetings**

02:10pm **Solution Provider Presentation Contact Steve Hambrook at steve.h@mnmconferences.com**

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

02:25pm **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

Dr.Sandra Engle, Research Associate, Pfizer,CT, **USA**

02:55pm **Panel Discussion: Making the cut: comparing the CRISPR- Cas9 system with ZFNs and TALENs**

- Comparing each technologies with respect to their accuracy
- Analyzing the latest advancement in each of these technologies
- Comparing the cost benefit ratios of these technologies

Dr. Philippe Duchateau, Chief Scientific Officer, **Cellectis, France**
Dr. John Feder, Associate Director of Genome Biology**, Bristol-Myers Squibb,NY,USA**
Dr. Sandra Engle, Research Associate, **Pfizer,CT,USA**

03:25pm **Solution Provider Slot; contact Steve Hambrook at steve.h@mnmconferences.com**

03:55pm **Afternoon Refreshment and Poster Presentation | One-to-One Networking Meetings**

04:35pm **Introduction to Cas9 nickase-induced precise genome engineering**

- Different sgRNA expression vectors, different lengths/permutations of sgRNA and use of sgRNA directly
- Use of RE site addition/deletion PAM, modifications to inhibit re-cutting Use of Cel1/Surveyor types of assays
- Transfection/introduction of reagents into hard to TX human cells and alterations of cellular repair machinery to enhance Cas9/CRISPR

Dr. Eric Hendrickson, Professor, **Harvard Medical School,MA,USA**

05:05pm **Workshop A: Understanding effective ways to overcome the delivery system in CRISPR**

Workshop Rational: CRISPR technology has gained an advantage over other technologies as it allows easy manipulation of DNA in the nucleus of any cell. However, there few challenges associated with this technology.

Benefits of Attending: This work shop gives you an opportunity to overcome its shortcoming and can develop gene models in precise manner so it is easy to predict the outcome.

Agenda:

- Effectiveness in dealing and understanding how to handle or stop the alteration of other gene other than the targeted gene.
- Evaluating approach for CRISPR technology that can be used for altering multiple gene and one time in a precise manner.
- Understanding CRISPR technology is precise to use on human gene editing.

06:05pm **Chairman's Closing Remarks**

06:10pm **Drinks Reception & Networking**

End of Conference Day1

Genome Editing & Engineering Conference

Day 2, Friday, February 19th

08:30 am **Registration & Refreshment**

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

09:00 am **Therapeutic Approach: Applying gene editing in T-cell immunotherapy**
- TALEN engineered T-cells
- Allogeneic CAR T-Cell based therapies
- Drug resistance
- Checkpoint inhibition

Dr. Philippe Duchateau, Chief Scientific Officer, Cellectis, **France**

09:30am **Solution provider's presentation: Keynote-2**

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

10:00am **Elucidating Telomere Function in Human Tumor Biology**
- Developing tools for genome editing in human pluripotent stem cells
- Using genome editing to test genome wide association studies (GWAS)
- Editing cancer associated mutations in stem cell models e.g. TERT promoter mutations
- Development of tissue specific cancer models using in vitro differentiation of edited cells

Dr. Dirk Hockemeyer, Principal Investigator, **University Of California-Berkeley, CA,USA**

10:30am **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, MA,USA**

11:00am **Morning Refreshment and Poster Presentation | One-to-One Networking Meetings**

11:40am **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**

12:10pm **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, **National Institutes of Health, NY, USA**

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

12:40pm **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**

01:10pm **Lunch and Poster Presentation | One-to-One Networking Meetings**

02:10pm **Solution Provider Slot; contact Steve Hambrook at steve.h@mnmconferences.com**

02:40pm **Genome editing in preclinical models for drug discovery**

- Use of ZFN/CRISPR for rapid generation of KO rodent models
- Use of Genome Editing tools to model human genetic mutation in preclinical models
- Use of Genome Editing tools to generate humanized models for drug discovery
- Challenges of Genome Editing Tools for model generation

Dr. Myung K. Shin, Associate Director, **Merck & Co, NY,USA**

03:10pm **Workshop B: Sequencing a mouse model and zebrafish model using CRISPR**

Workshop Rational: The mouse and zebrafish are powerful and easy to handle animal models. These animal models are used for functional genomics analysis, study of human disease pathogenesis and also for the discovery and development of new drugs. Here at the conference we focus on how to develop mouse and zebrafish genetic sequence model using CRISPR technology.

Benefits of Attending: The attendee would be able to understand the hands on detailed procedures required while developing animal model sequence using CRISPR technology.

Agenda:

- Understanding the effectiveness of using CRISPR technology in animal model
- Case study on developing animal model sequencing and the success story
- Which animal model is more accurate in genome editing for drug discovery and developing new drugs

04:10pm **Closing remark from chair**

04:15pm **End of Conference**

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