

# 2<sup>nd</sup> Annual Genome Editing & Engineering Conference

Devising precision gene editing & engineering: CRISPR-Cas9, Gene therapy, RNAi

6 - 7 February 2017, San Diego-CA, USA

## Advisory Committee:



**Jacques P Tremblay**  
Full Professor, Department of  
Molecular Medicine  
University of Laval



**C.B. Gurumurthy**  
Director, Transgenic Core Facility  
University of Nebraska Medical  
Center

## Event Overview:

Genome editing holds the promise of progressing in the area of pharmaceutical science since the discovery of the CRISPR/Cas systems.

The 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.

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 integration of genome, off-target effects, and delivery systems.

With the tremendous response on our past annual Genome Editing & Engineering Conference 18th-19th February 2016, we are excited to introduce the **2nd Annual Genome Editing & Engineering Conference, 6th-7th February 2017 in San Diego.**

## Key Highlights

- **RNA guided nuclease** for genome modifications
- Different **gene delivery systems**
- **CRISPR** as a molecular tool for **programmable gene expression** and **gene therapy**
- Gene **knock in** and genomic screening using **TALEN** and **CRISPR**
- CRISPR **biomedical research** applications
- **RNAi based** screening technologies
- Genome editing for **disease modeling**

## Who should attend?

*From Pharmaceutical and Bio-pharmaceutical companies:*

**Chief Scientific Officer/ Directors/ Heads/ VP/ Senior Scientists/ Principal Scientist/ Project Leader in:**

- ✓ Genome biology
- ✓ Functional genomics
- ✓ Gene/Cell therapy
- ✓ Genome/Genetic Engineering
- ✓ Genetics
- ✓ Molecular Biology
- ✓ Drug discovery
- ✓ Therapeutics
- ✓ Regenerative medicines
- ✓ Translational sciences
- ✓ Immunology
- ✓ Immuno-oncology

*From Universities and Research institutes:*

**Professors/ Assistant Professors/ Researchers/ Scientists/ Principal Scientists in:**

- ✓ Genomic/Genetic Engineering
- ✓ Genetics
- ✓ Functional Genomics
- ✓ Genome Biology
- ✓ Gene Editing/ Genome Editing
- ✓ Molecular Biology
- ✓ Bio-molecular Engineering
- ✓ Bio-chemistry
- ✓ Regenerative medicines
- ✓ Stem cell
- ✓ Gene Therapy/Cell Therapy
- ✓ Immunology
- ✓ Immunotherapy

For more information please contact Ajay at  
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## Expert Speaker Panel:



**Dana Carroll**  
Distinguished Professor,  
Department of Biochemistry  
**University of Utah School  
of Medicine (Originator of  
ZFN), Utah**



**Jacques P Tremblay**  
Full Professor, Department of  
Molecular Medicine  
**University of Laval, Canada**



**Daniel Anderson**  
Associate Professor,  
Department of Chemical  
Engineering  
**Massachusetts Institute of  
Technology, Massachusetts**



**C.B. Gurumurthy**  
Director, Transgenic Core  
Facility  
**University of Nebraska  
Medical Center, Nebraska**



**Niren Murthy**  
Professor in the Department  
of Bioengineering  
**University of California  
at Berkeley, Berkley,  
California**



**Shondra Miller**  
Director, Genome  
Engineering and iPSC  
Center (GEIC), **Washington  
University School of  
Medicine, Washington**



**Ite A. Laird-Offringa**  
Director, Programs in  
Biomedical and Biological  
Sciences **University of  
Southern California,  
California**



**Benjamin Borgo**  
Product Manager  
**Agilent Technologies,  
California**



**Devon Shedlock**  
Director, Immunooncology  
**Poseida Therapeutics,  
California**



**Deepak Rajpal**  
Director, Computational  
Biology  
**GlaxoSmithKline,  
Pennsylvania**



**Tetsushi Sakuma**  
Assistant Professor,  
Molecular Genetics  
laboratory  
**Hiroshima University, Japan**



**Raman Sood**  
Head, Zebrafish Core,  
National Human Genome  
Research Institute  
**National Institutes of  
Health, Maryland**



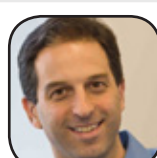
**Wu Haoquan**  
Associate Professor,  
Infectious Diseases  
**University of Texas, Texas**



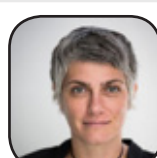
**Michael Kyba**  
Professor, Department of  
Pediatrics  
**University of Minnesota,  
Minnesota**



**Theodore Friedmann**  
Professor, Institute of  
Engineering in Medicine  
**University of California, San  
Diego, California**



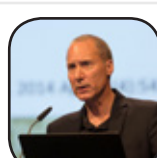
**Ron Weiss**  
Professor of Biological  
Engineering  
**Massachusetts Institute of  
Technology, Massachusetts**



**Cecilia Cotta-Ramusino**  
Senior Scientist  
**Editas Medicine,  
Massachusetts**



**Imran Noorani**  
Wellcome Trust Sanger  
Institute  
**Cambridgeshire,  
United Kingdom**



**Nicholas J. Schork**  
Director Human Biology  
**J. Craig Venter Institute  
(JCVI), California**



**Mark Dewitt**  
Research Fellow  
**Innovative Genomics  
Initiative, Berkley, California**

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## **DAY 1, 6<sup>th</sup> February 2017**

**08:00 Registration**

**08:55 Welcome note from Marketsandmarkets**

**09:00 Opening Remarks from the Chairman**

**09:10 Keynote Presentation - Nucleic acid delivery systems for RNA therapy and gene editing**

- High throughput, combinatorial approaches have revolutionized small molecule drug discovery.
- Libraries of degradable polymers and lipid-like materials have been synthesized, formulated and screened for their ability to deliver RNA, both in vitro and in vivo.
- A number of delivery formulations have been developed with in vivo efficacy, and show potential therapeutic application for the treatment of genetic disease, viral infection, and cancer.

**Daniel Anderson**, Associate Professor, Department of Chemical Engineering, **Massachusetts Institute of Technology, Massachusetts**

### **Novel Targeted Genome Editing**

**09:35 Everything is CRISPR with Targeted Deep Sequencing: Using Targeted Deep Sequencing to Better Design Your CRISPR Experiments**

- Comparison of different assays that determine gRNA activity (T7E1 vs. TIDE vs. NGS)
- Discuss how next generation sequencing can be used to screen for modified clones and/or mosaic animals with the desired modification
- Demonstrate how next generation sequencing can help investigators rapidly and accurately design their genome editing experiments to reduce overall labor and expense.

**Shondra Miller**, Director, Genome Engineering and iPSC Center (GEIC), **Washington University, School of Medicine, Washington**

**10:00 Easi-CRISPR and other methods for efficient knocking-in of large DNA inserts**

- Easi-CRISPR; a highly efficient method for developing knock-in and conditional knockout animal models
- Latest strategies for increasing HDR efficiencies

**C.B. Gurumurthy**, Director, Transgenic Core Facility, **University of Nebraska Medical Center, Nebraska**

*10:25 Solution Provider Presentation; Please contact at [steve.h@marketsandmarkets.com](mailto:steve.h@marketsandmarkets.com)*

**10:55 Morning Refreshments | Poster Presentations | One-to-One Networking**

**11:40 Highly practical gene knock-in in mammalian cells and zygotes with MMEJ-dependent strategy**

- Development of PITCH (Precise Integration into Target Chromosome) system
- Highly efficient selection-free PITCH knock-in (>20%) in human cells
- Highly practical PITCH knock-in in mouse zygotes by combining cloning-free CRISPR/Cas

**Tetsushi Sakuma**, Assistant Professor, Molecular Genetics laboratory, **Hiroshima University, Japan**

**12:05 High throughput genome editing in zebrafish for functional genomics and disease modeling**

- Use of zebrafish as a model for functional genomics and disease modeling
- High throughput methods for genome editing using CRISPR/Cas9
- Examples to demonstrate phenotype of the mutant fish

**Raman Sood**, Head, Zebrafish Core, National Human Genome Research Institute, **National Institutes of Health, Maryland**

*12:30 Solution Provider Presentation; Please contact at [steve.h@marketsandmarkets.com](mailto:steve.h@marketsandmarkets.com)*

**13:00 Lunch | Poster Presentations | One-to-One Networking**

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## 14:00 Panel Discussion: Viral gene delivery System vs. Non-viral gene delivery system

- Different types of vectors
- Advantages and disadvantages
- Applications

**Niren Murthy**, Professor, Department of Bioengineering, **University of California, California**

## Therapeutic Genome Editing

### 14:30 Use of the CRISPR/Cas9 system to correct mutations responsible for Duchenne Muscular Dystrophy and Friedreich ataxia

- Using 2 gRNAs targeting the exons that precede and follow the DMD patient deletion it is possible to form a hybrid exon that restore the normal reading frame.
- Using 2 gRNAs targeting the exons that precede and follow the DMD patient deletion it is possible to form a hybrid exon such that the dystrophin protein produced contains correct spectrin like repeats.
- It is possible to remove the abnormal GAA repeat in intron 1 of the frataxin gene in Friedreich patients by inducing double strand breaks with 2 gRNAs targeting before and after that repeat

**Jacques P Tremblay**, Full Professor, Department of Molecular Medicine, **University of Laval, Canada**

### 14:55 Combining protein and genome editing to analyze in-genome transcriptional regulation of lowly expressed genes

- Constructing functional protein fusions
- Luciferase fusion reporter
- CRISPR genome engineering of immortalized primary cells

**Ite A. Laird-Offringa**, Director, Programs in Biomedical and Biological Sciences, **University of Southern California, California**

### 15:20 In-vivo delivery of Cas9 protein, guide RNA and template DNA to generate HDR

- Rescue mice from Duchenne muscular dystrophy
- Low off-target DNA mutations

**Niren Murthy**, Professor, Department of Bioengineering, **University of California at Berkeley, California**

**15:45 Solution Provider Presentation; Please contact at [steve.h@mnmcconferences.com](mailto:steve.h@mnmcconferences.com)**

## 16:00 Evening Refreshments | Poster Presentations | One-to-One Networking

### 16:45 Piggy-bac transposon based forward genetic screens in mice for tumors of the nervous system:

- Using this transposon system, we are generating large numbers of tumors of the eye and brain in transgenic mice
- Next-generation sequencing of the tumors will identify common insertion sites of the transposon.
- This approach will highlight driver genes of nervous system tumors in vivo

**Imran Noorani**, Medical Doctor, **Wellcome Trust Sanger Institute, United Kingdom**

### 17:00 Workshop: SureGuide CRISPR Libraries for Functional Genomics Screening: Do You Know What's in Your Libraries?

- Demonstrate improved library synthesis and superiority of SureGuide in comparison with competing technologies
- Discuss the development of novel chemistries, a novel, overlap-based cloning method and workflows necessary for generating high quality, low-bias plasmid libraries
- Discuss on-going CRISPR projects and new developments at Agilent

**Benjamin Borgo**, Product Manager, **Agilent Technologies, California**

### 17:50 Closing Remarks by the Chair

### 18:00 End of Day 1

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## DAY 2, 7<sup>th</sup> February 2017

08:15 Registration

08:55 Welcome note from Marketsandmarkets

09:00 Opening Remarks from the Chairman

09:10 Keynote Presentation – **Issues in Programmable Genome Editing**

- There are several available genome editing platforms.
- Editing outcomes depend on cellular DNA repair activities
- One approach to a clinical application of CRISPR

**Dana Carroll**, Distinguished Professor, Department of Biochemistry, **University of Utah School of Medicine, Utah**

### Therapeutic Applications of Genome Editing

09:35 **Gene therapy and genome editing**

- Clinical advances of gene therapy
- Genome editing and the changing face of gene therapy
- Genetic enhancement and ethical and social dilemmas posed by human genetic modification

**Theodore Friedmann**, Professor, **Institute of Engineering in Medicine, University of California, California**

10:00 **Disease Signatures in Drug Discovery**

- Application of clinical transcriptomics to derive disease transcriptomics
- Understanding Reversal Signatures
- Applications in drug discovery development strategies

**Deepak Rajpal**, Director, Computational Biology, **GlaxoSmithKline, Pennsylvania**

10:25 *Solution Provider Presentation; Please contact at [steve.h@marketsandmarkets.com](mailto:steve.h@marketsandmarkets.com)*

10:55 **Morning Refreshments | Poster Presentations | One-to-One Networking**

11:40 *Solution Provider Presentation; Please contact at [steve.h@marketsandmarkets.com](mailto:steve.h@marketsandmarkets.com)*

11:55 **Optimizing sgRNA structure to improve CRISPR-based genome-wide knockout screening performance**

- Optimizing sgRNA structure to improve knockout efficiency
- Optimized sgRNA can improve the performance of genome-wide screening
- Identifying virus host factors using CRISPR-based knockout screening

**Wu Haoquan**, Associate Professor, Infectious Diseases, **University of Texas, Texas**

12:20 **IMammalian Synthetic Biology: From Parts to Modules to Therapeutic Systems**

- Recent advances in synthetic biology for engineering genetically encoded live cell sensory and regulatory devices. These devices allow us to sense in living cells various important cell biomarkers, including miRNA and protein levels, and to create logic circuits that identify complex profiles for cell state and respond accordingly.
- Two areas for deploying sensing/actuation genetic circuits include programmable organoids and cancer immunotherapy. Our genetic circuits provide real time single cell analysis and response that can be beneficial for drug development using organoids and for automated multi-step immunomodulatory cancer therapies.

**Ron Weiss**, Professor of Biological Engineering, **Massachusetts Institute of Technology, Massachusetts**

12:45 **Genomic profiling for single cell and therapeutic cell replacement analyses**

- Single cells, even within a small confined area of a tissue, are often heterogeneous at the genomic level
- Characterizing genomic heterogeneity at the single cell level is crucial to understanding how molecular pathologies arise and how these pathologies might be treated with cell replacement strategies
- Cells created in vitro for therapeutic cell replacement purposes must be vetted at the genomic and other levels to ensure their safety, which is not trivial
- Designing studies to exploit cell replacement therapies can leverage genomic profiling

**Nicholas J. Schork**, Director Human Biology, **J. Craig Venter Institute (JCVI), California**

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**13:10 Lunch | Poster Presentations | One-to-One Networking**

**14:10** *Solution Provider Presentation; Please contact at [steve.h@marketsandmarkets.com](mailto:steve.h@marketsandmarkets.com)*

**14:25 Using NextGEN™ CRISPR for Safe and Efficient Genome Editing of CAR-T cells**

- Develop NextGEN™ CRISPR site-specific nucleases (NG) and optimize genome editing in resting T cells
- Enhance potency of autologous CAR-T therapies by targeting inhibitory receptors
- Use NG to produce 'universal' allogeneic CAR-T cells

**Devon Shedlock**, Director, Immunooncology, **Poseida Therapeutics, California**

**14:50 Panel Discussion: CRISPR vs. RNAi-based screening technologies**

- Different approaches and strategies
- Functional studies
- Advantages and Disadvantages

**15:15 Evening Refreshments | Poster Presentations**

**15:50 FSHD: approaches to genetically correct cells carrying a dominant 100+ kb deletion mutation**

- FSHD is a dominant disease caused by mutations that reduce copy number of a 3.3 kb repeat near the telomere of chromosome 4
- Because it is dominant, loss of function approaches, such as gene editing the poly A signal outside of the repeat are feasible.
- Because it is subtelomeric, deleting the terminus of chromosome 4 by inserting a new telomere can also be considered

**Michael Kyba**, Professor, Department of Pediatrics, **University of Minnesota, Minnesota**

**16:15 Interconnection between Cas9 variants and DNA repair pathways**

- Different Cas9 variants generate different lesions
- These lesions are sensed and repaired by different mechanisms
- Understanding the differential pathway engagement allows for a more deterministic approach in designing research and therapeutic genome engineering strategies

**Cecilia Cotta-Ramusino**, Senior Scientist, **Editas Medicine, Massachusetts**

**16:40 Gene Editing Approaches to the Treatment of Sickle Cell Disease**

- Gene has opened a new front in the treatment of genetic blood disease, in particularly sickle cell disease (SCD).
- There are a variety of approaches. Perhaps the most desirable is HDR-mediated correction of the sickle mutation itself.
- I will focus on our work correcting the SCD mutation in human bone marrow stem cells, and contrast our approach to those of others in the field.

**Mark Dewitt**, Research Fellow, **Innovative Genomics Initiative, Berkeley, California**

**17:05 End of Conference**

## Media Partners



For more information please contact Ajay at  
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