



4th Annual Genome Editing & Engineering Conference

7th & 8th February 2019 · San Diego-CA

Event Overview

Over the years, gene editing has evolved and marked its presence in therapeutics and drug discovery through specific gene targeting and animal modeling based clinical trials.

The industry of genome editing has witnessed some great breakthroughs during a year in the areas like genome writing, base editing and specific progression in the field of therapeutics through the Car-T cells and immunotherapy.

To gather such insights and more, we welcome you once again to our **4th Annual Genome Editing & Engineering Conference**, on **8th & 9th February 2019** in **San Diego, CA**.

For more information please contact Amit at
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Key Highlights

- Base editing through genome editing tools
- Target efficiency
- Homology directed repair mutations
- Ethical issues for gene therapy
- Precision gene editing
- Diagnosis of therapy through CRISPR
- Single cell base editing
- Car-T cell therapy
- Sickle cell disease treatment

Who should attend?

From Pharmaceutical and Bio-pharmaceutical companies:

Chief Scientific Officers/ CEOs/Senior Scientists/ Principal Scientists/ Project Leaders/Heads in:

- Genome biology
- Functional genomics
- Gene/Cell therapy
- Genome/Genetic Engineering
- Genetics
- Molecular Biology
- Translational sciences
- 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
- Gene Therapy/Cell Therapy
- Immunology
- Immunotherapy

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Advisory Committee

- **C.B. Gurumurthy**, Director Transgenic Core Facility, **University of Nebraska, USA**
- **Aaron Cheng**, Head, PTS Discovery Genome Editing, **GSK, USA**
- **Farren Issac**, Principal Investigator, **Yale University, USA**
- **Danilo Madillo**, Laboratory Head, **Novartis, Switzerland**

Expert Speaker Panel

- **C.B. Gurumurthy**, Director Transgenic Core Facility, **University of Nebraska, USA**
- **Aaron Cheng**, Head, PTS Discovery Genome Editing, **GSK, USA**
- **Farren Issac**, Principal Investigator, **Yale University, USA**
- **Danilo Madillo**, Laboratory Head, **Novartis, Switzerland**
- **Nitsch Roberto**, Associate Director, **AstraZeneca, UK**
- **Theodore Friedmann**, Professor, Pediatrics, **UC San Diego, US**
- **Norbert O. Reich**, Principal Investigator, Reich Group, **University of California Santa Barbara, CA**
- **Raman Sood**, Director, Zebrafish core, **NIH- National Human Genome Research Institute, MD**
- **Xianghong Li**, Associate Director, Gene therapy R&D, **Poseida Therapeutics, CA**
- **Miguel Foronda**, Research Associate, Dow Lab, **Weill Cornell Medicine, NY**
- **Elizabeth Garner**, Senior Scientist, **Caribou Biosciences, CA**
- **Luca Pinello**, Assistant Professor, Pinello Lab, **Massachusetts General Hospital, MA**
- **Niren Murthy**, Professor, Bioengineering, **University of California Berkley, CA**
- **Ram Kannan**, Research Scholar, Ventura's Laboratory, **Memorial Sloan Kettering Cancer Center, NY**
- **Gene Yeo**, Professor of Cellular and Molecular Medicine, **University of California-San Diego, CA**
- **Laurent Poirot**, Head of Early Discovery, **Collectis, France**

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DAY 1, Thursday, 7th February 2019

- 07:55 Registration
- 08:45 Keynote by MarketsandMarkets
Shailender Singh (Shelly), COO, MarketsandMarkets
- 08:55 Opening remark by Chairman

TECHNOLOGICAL DEVELOPMENTS IN NUCLEASES

- 09:00 Cas9-assisted gRNA-free one-step genome editing with no sequence limitations and improved targeting efficiency
- 09:25 CRISPR to induce large amount of edits
- 09:50 Solution provider presentation
- 10:20 Morning refreshment and Networking
- 11:10 Base editors to conduct genome editing without double-stranded breaks
- 11:35 Application of POBEC3A-Cas9 base editor to minimize bystander and off-target activities
- 12:00 Strategies to improve the rate of CRISPR-Cas9 rate of Homology Directed Repair
- 12:25 Solution provider presentation
- 12:55 Lunch & Networking

PRECISION GENE EDITING

- 13:55 Zinc Finger Nuclease mediated precision genome editing for DNA repair
- 14:20 Evolution of human pluripotent stem cells by precise genome editing
- 14:45 Application of genome editing technology to disease modeling in zebrafish
- High throughput methods for CRISPR/Cas9 mediated mutagenesis in zebrafish
 - Unexpected consequences of CRISPR/Cas9 induced indel mutations
 - Base editing and homology-directed repair for targeted knockin
- Raman Soodm, Director, Zebrafish core, NIH- National Human Genome Research Institute, MD
- 15:10 Solution provider presentation
- 15:25 Afternoon Refreshment & Networking
- 16:15 Solution provider presentation

CONTROVERSIES OF GENOME EDITING

- 16:30 CRISPR might induce DNA damage?
- 16:55 Can CRISPR cause Cancer?
- 17:20 Closing remark by Chairman
- 17:25 Drink Reception & Networking
- 18:25 End of Day1

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DAY 2, Friday, 8th February 2019

08:30 Registration

08:55 Opening remark by Chairman

COMMERCIALIZATION OF GENOME EDITING

09:00 **Progress in Gene therapy**

- Gene therapy has become a clinical and a commercial reality and soon will represent the standard of care in some genetic disorders
- Conceptual and technical advances including a combination of immunotherapy and gene therapy (CAR-T techniques) are making previously intractable diseases including as a growing list of cancers amenable to effective therapy
- Genome editing approaches are rapidly being applied to prevent and treat disease, but a serious potential exists for premature and inappropriate human germ-line modification for unwise enhancement purposes

Theodore Friedmann, Professor, Pediatrics, **UC San Diego, US**

09:25 **Diagnosis and therapy through CRISPR based tools for point of care testing and early therapies**

09:50 [Solution provider presentation](#)

10:20 **Morning refreshment and Networking**

11:00 **Single cell base editing through allele specific CRISPR**

11:25 **Targeted genome editing through RNA guided Cas9**

11:50 [Solution provider presentation](#)

12:15 **Treatment of autosomal disorders through in-vivo delivery of genome editing agents**

12:40 **Lunch & Networking**

13:40 **Editing efficiencies improvement in T-cells which is divided in 3 types: Actual therapeutics (manufacturing), Single target editing, Pre-clinical stage**

14:05 **Car-T space combinational therapy**

14:30 **Gene therapy for the cure of sickle cell disease**

14:55 **Modeling oncogenic Braf duplications in mice using genome editing**

- Employed CRISPR to generate Kiaa1549-Braf tandem duplication ex-vivo in adult neural stem cells
- Kiaa1549-Braf driven tumors exhibit features of diffuse leptomeningeal glioneuronal tumor
- Kiaa1549-Braf driven MAPK signaling is inhibited by preclinical drug BGB3245

Ram Kannan, Research Scholar, Ventura's Laboratory, **Memorial Sloan Kettering Cancer Center, NY**

15:20 **Afternoon Refreshments & end of conference**