Genome Editing Applications

Therapeutic and Biomedical Applications of CRISPRs, ZFNs, TALENs and other Genome Editing Technologies

Wednesday 2 - Thursday 3 December 2015
Sheraton Airport Hotel, Brussels, Belgium

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TJ Cradick
CRISPR Therapeutics
USA

Philippe Duchateau
Cellectis
France

Myung Shin
Merck & Co., Inc.
USA

Ines Royaux
Janssen R&D, a division of Janssen Pharmaceutica NV,
Belgium

Lorenz Mayr
AstraZeneca
UK

William Skarnes
The Wellcome Trust Sanger Institute, UK

Charles Gersbach
Duke University
USA

Victor Bartsevich
Precision Biosciences, Inc.
USA

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www.informa-ls.com/genomeediting

Day One: Wednesday 2nd December 2015

08:00 **Registration and Morning Coffee**

08:50 **Chairperson's Opening Remarks**

KEYNOTE OPENING ADDRESS: FUTURE OF GENOME EDITING

09:00 **Thought Leaders Discussion:**
Evaluating Current Genome Editing Trends and Strategies for Unlocking Future Applications

- The future of genome editing: What has been achieved and what does the future hold?
- Application of genome editing as a therapeutic: What makes a good genome editing target?
- What platform advancements are necessary to significantly broaden the range of CRISPR-based therapeutics?
- How best to deploy genome editing reagents, control expression and delivery of editing
- Translating gene editing *in vivo* and into the clinic

TJ Cradick, Head of Genome Editing, **CRISPR Therapeutics,** USA
Lorenz Mayr, Vice President & Global Head, Biological Reagents & Assay Development, **AstraZeneca,** UK
Philippe Duchateau, Chief Scientific Officer, **CELLECTIS SA,** France
Charles Gersbach, Associate Professor, **Duke University,** USA

GENOME EDITING APPLICATIONS IN DRUG DISCOVERY AND SCREENING

09:35 **Transforming and Translating Drug Discovery – Use of the CRISPR/Cas9 Technology in Target Discovery, Hit Finding and Translational Studies**

This presentation will describe the use of CRISPR/Cas9 technology for target-validation studies in our *in vitro* and *invivo* disease models at AstraZeneca. Furthermore, we will also describe our activities with in-house programs and strategic external partnerships towards genome wide target discovery and target validation studies.

Lorenz Mayr, Vice President & Global Head, Biological Reagents & Assay Development, **AstraZeneca,** UK

10:05 **Genome Editing Applications in Drug Discovery and Screening**

In order to speed up the drug discovery process, Janssen R&D has adopted gene editing technologies across its drug discovery pipeline. Here we will show how those technologies can contribute to the generation of better cellular and animal models as well as "cleaner" CRISPR-based genetic screens for target validation/identification. We will also present our first efforts to use genome editing for ex vivo gene therapy.

Ines Royaux, Senior Scientist, **Janssen R&D, a division of Janssen Pharmaceutica NV,** Belgium

10:35 Morning Coffee and Networking

11:05 **Enhancing Functional Genomics and Screening through the Application of Genome Editing Technologies**

New developments in gene-editing technologies such as CRISPR-CAS9 can potentially improve the output and interpretation of large scale screening results. A comparison of the different gene-editing technologies in large scale functional genomic screening will be discussed.

Roderick Beijersbergen, Group Leader and Head NKI Robotics and Screening Center, **The Netherlands Cancer Institute,** The Netherlands

11:35 **Chemically Modified Guide RNAs Enhance CRISPR-Cas Genome Editing in Human Primary Cells**

Using a recent advancement in RNA synthesis technology, we find it straightforward to chemically synthesize RNAs of more than 100 nt in length and to incorporate modified nucleotides at any position. We have demonstrated that chemical alterations to synthesized single guide RNAs (sgRNAs) enhance genome editing efficiency in human primary T cells and CD34+ hematopoietic stem and progenitor cells. This approach is a simple and effective way to streamline the development of genome editing with the potential to accelerate a wide array of biotechnological and therapeutic applications of the CRISPR-Cas technology.

Laurakay Bruhn, Section Manager Biological Chemistry, **Agilent Research Labs,** USA

12:05 **Application of CRISPR/Cas9**

For more information on this presentation please visit the event website www.informa-ls.com/genomeediting

Helene Faustrup Kildegaard, Co-Principal Investigator, **The Novo Nordisk Foundation Center for Biosustainability, Technical University of Denmark,** Denmark

12:35 **CRISPR and RNAi Platforms for Genome-Wide Loss-of-Function Genetic Screens**

Genome-wide loss-of-function pooled screens provide a direct approach to identify genes regulating biological responses and find new therapeutic targets. While RNAi screens have proven effective, CRISPR/Cas9 provides a newer attractive alternative. To complement our established shRNA screening platform, we have developed similar pooled sgRNA libraries for functional CRISPR knockout screens and ran screens in PDX-derived cell lines to compare the performance of each.

Paul Diehl, Director of Business Development, **Cellecta, Inc.** USA

13:05 Lunch and Live Labs Exhibition Tour with Cell and Gene Therapy Meeting

APPLICATION FOR CELL LINE ENGINEERING AND THE GENERATION OF MORE PREDICTIVE DISEASE MODELS

14:30 **Generation and Characterisation of a Superior CHO Host Cell Line for Biomanufacturing using Gene Editing Technologies**

Functional transcriptome analysis of Chinese hamster ovary cell lines identified the loss of a telomeric region of chromosome 8 in high producing cells. Deleting relevant genes of this region using cell line engineering tools led to new parental cell lines, which display several advantageous characteristics for cell line development and manufacturing of recombinant therapeutics decreasing cost and time efforts.

Anett Ritter, Postdoctoral Fellow, **Novartis Pharma AG,** Switzerland

15:00 **Spotlight Presentation**

This presentation will be hosted by a leading company who operates in the field of genome editing. **Please contact: linda.cole@informa.com +44 (0) 20 701 76631**

15:30 Afternoon Tea and Networking Time

16:10 **Application of ZFN/CRISPR to Generate High Efficiency, Throughput and Predictive Animal Models for Drug Discovery**

ZFN and CRISPR have allowed for rapid generation of genetically engineered models in various preclinical species. We will present how these tools have been applied to efficiently generate various animal models including knockout, knock-in and large deletion mutants. Furthermore, we will highlight the application of these models in drug discovery including studying human genetic findings and drug metabolism.

Myung Shin, Senior Principal Scientist, **Merck & Co., Inc.** USA

16:40 **Ex Vivo Genome Editing: Editing of Stem Cells to Generate Translational Disease Models**

Human induced pluripotent stem cells (hiPSCs) are an ideal model cell for the study of basic cellular and developmental processes and for modelling human disease. Using CRISPR technology, my lab has developed efficient, scalable methods for biallelic modification of hiPSCS to generate isogenic pairs of mutant and revertant cell lines.

William C. Skarnes, Senior Group Leader, **Wellcome Trust Sanger Institute,** UK

17:10 **Closing Remarks from the Chairperson**

17:30 **Drinks and Networking Reception**

18:15 **Evening Seminar, Discussion and Dinner:**
Intellectual Property and Business Strategy Landscape of Genome Editing Technologies

Day Two: Thursday 3rd December 2015

MANUFACTURING GENETICALLY MODIFIED T-CELLS

08:55 Chairperson's Opening Remarks

09.00 **The ExCELLence Cell Handling Platform for Efficient Production of Gene-Modified T-Cells**

Lothar Germeroth, SVP, Managing Director, **Juno Therapeutics GmbH,** Germany

09.20 **Manufacturing Strategies for CAR-T Therapies –Cellectis Case Study**

Sylvain Arnould, Head, Manufacturing, **Cellectis,** France

09.40 **Simplify, Optimise and Control – The Autolus Approach to CAR-T Manufacturing**

Jim Faulkner, Head, Manufacturing, **Autolus,** UK

10.00 **DISCUSSION PANEL: The Realities of Manufacturing CAR-T Cells**

- What are the unique manufacturing strategies for CAR-T cells?
- In-house vs. external manufacturing of CAR-T cells
- Centralised vs. de-centralised models
- Vector development issues
- Supply chain management of CAR-T cells
- Workflow optimisation in a commercial phase overlaps between process development and quality
- Software support for CAR-T cells

Lothar Germeroth, SVP, Managing Director, **Juno Therapeutics GmbH,** Germany

Bernadette Keane, VP, QA/QP, **Bluebird,** USA

Gwendolyn Binder- Scholl, Executive VP, **AdaptImmune,** USA

Sylvain Arnould, Head, Manufacturing, **Cellectis,** France

Jim Faulkner, Head, Manufacturing, **Autolus,** UK

10:30 Morning Coffee and Networking

IDENTIFYING AND MANAGING OFF-TARGET ACTIVITY

11:00 **Bioinformatics for CRISPR Activity and Minimising Off-Target Activity**

Gene therapy applications for nucleases require greater concern for optimising on-target activity and minimising possible off-target effects, though off-target cleavage impedes both research and therapeutic use. Bioinformatics programs are essential for guiding the choice of nucleases and evaluating if off-target cleavage events have occurred. PCR and sequencing assays make this straight forward for many labs and allow optimisation of nucleases, delivery and cellular treatments.

TJ Cradick, Head of Genome Editing, **CRISPR Therapeutics,** USA

11:30 **Designing for Success with Genome Editing Software**

For more information on this presentation please visit the event website www.informa-ls.com/genomeediting

Victor Dillard, COO & Founder
Desktop Genetics, UK

THERAPEUTIC APPLICATIONS OF GENOME EDITING

12:00 **Applying TALEN-Based Gene Editing to Improve CAR T-Cells for Adoptive Immunotherapy**

CAR engineered autologous T cell therapy has shown promising efficacy in B cell malignancies. Similarly, transcription activator-like effector nuclease (TALEN™)-mediated gene editing has emerged as powerful strategy to introduce targeted mutations and holds great promise in therapeutics and offers multiple opportunities to improve CAR T-cell therapies.

Philippe Duchateau, Chief Scientific Officer, **CELLECTIS SA,** France

12:30 **Genome and Epigenome Editing for Treating Genetic Diseases and Programming Cell Phenotype**

The advent of genome engineering technologies, including the RNA-guided CRISPR/Cas9 system, has enabled the precise editing and regulation of endogenous human genes and epigenetic states. In this presentation I will discuss our work on applying these tools to the correction of mutations that cause genetic disease, such as Duchenne muscular dystrophy. Additionally, I will present our work on adapting these tools to manipulating the epigenome and their application to controlling cell fate decisions.

Charles Gersbach, Associate Professor, **Duke University,** USA

13:00 **Spotlight Presentation**

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13:15 Lunch and Networking with Cell and Gene Therapy Meeting

13:45 **Optional Lunchtime Interactive Roundtable Discussion: Improving Delivery of Genome Editing**

14:15 **Meganucleases as an Efficient Tool for Genome Editing**

Unlike ZFNs, TALENs, and CRISPRs, engineered meganucleases have largely remained behind the scenes due to difficulties in modifying their DNA-recognition specificity. We have developed a next-generation meganuclease platform called "ARCUS" that overcomes these production difficulties and can readily produce meganucleases with customised activity and specificity. We will present examples of successful application of the technology in gene and cell therapies.

Victor Bartsevich, Director of Genomic Editing Technology Development, **Precision Biosciences, Inc.** USA

14:45 **Gene Therapy Case Study: Application of Genome Editing**

Thierry VandenDriessche, Director of the Department of Gene Therapy and Regenerative Medicine **Vrije Universiteit Brussel** and Member Committee of Advanced Therapeutics, **European Medicines Agency,** Belgium

15:15 **Feedback from Amgen on the Application of Genome Editing**

Vidya Prabhu, Senior Scientist, Therapeutic Discovery, **Amgen,** USA

15:45 Afternoon Break and Networking

16:15 **Rescue of T-Cell Deficiency by Ex Vivo Haematopoietic Stem Cell Gene Editing and Transplantation in Prkdc Mice**

Rafael J. Yáñez-Muñoz
Reader in Advanced Therapy
Royal Holloway-University of London, UK

16:45 **Genome Engineering for a Novel Therapeutic Application**

Mitchell Finer
Former Chief Scientific Officer
Bluebird Bio, USA

TRANSLATING GENOME EDITING AND CRISPR THERAPEUTICS IN THE CLINIC

17:15 **Interactive Panel Discussion: Regulatory and Technical Strategies for Advancing Genome Editing Therapies into the Clinic**

- Current status and strategies for advancing genome editing and CRISPR therapeutics into the clinic
- What kind of models, data, safety and toxicity testing required to advance genome editing therapeutics into the clinic?
- Industry feedback on the path to the clinic for genome editing therapeutics
- Similarities and differences between EU and USA requirements

Panellists include:

Julian Hitchcock, Counsel, **Denoon Legal,** UK

17:35 End of Genome Editing Applications Conference

Accelerate your Therapeutics to Market and Improve Drug Discovery Efficiencies

Pre-Conference Workshop: Tuesday 1st December 2015

Innovative Approaches to Characterise and Enhance the Specificity of CRISPR/Cas9 Systems

Registration 10:00 – Start 10:30 – End no later than 16:30. Refreshments and lunch will be provided.

This full day workshop will focus on exploring innovative approaches for defining and improving the specificity of CRISPR/Cas9 systems.

Those who attend will benefit from an interactive format covering a range of topics including:

- Latest efforts to define, study and characterise the function and specificity of CRISPR
- Strategies to modify, improve and overcome the main challenges surrounding CRISPR specificity
- Monitoring editing methods: Understanding and monitoring what is happening at the target site of editing
- Methods to define off-target effects of CRISPR/Cas9
- Harnessing bioinformatics

Workshop Chairman:

TJ Cradick
Head of Genome Editing
CRISPR Therapeutics
USA

Evening Seminar, Discussion and Dinner: Wednesday 2nd December 2015

Intellectual Property and Business Strategy Landscape of Genome Editing Technologies

Registration 18:15 – Start 18:30 – End no later than 20:30. Networking Dinner to Follow

This interactive evening seminar will discuss the current status of the IP landscape of CRISPR/Cas9 and other genome editing technologies.

Topics to be discussed include:

- Current status of the IP landscape of CRISPR/Cas9 and other technologies
- What can you patent in the CRISPR area (e.g. genes, cell-lines, methods of treatment, etc)
- What other people are patenting (i.e. Freedom to Operate issues, Dounda patent applications, etc.)
- Timeframes expected for decisions to come out
- Considerations and negotiations for research, commercial, genome wide screening, applications and invention patents
- What happens if you apply for a research license and discover something which could be used for commercial use?
- Overview of the current business and partnering landscape in genome editing

Seminar Leader:

Philip Webber
Partner
Dehns (Patent and Trade Mark Attorneys)
UK

Present a Poster

You must be booked on as a delegate to be able to present a poster. To apply please send your abstract of 200 words or less, written in English, listing the principle author and all contact details to catherine.marshall@informa.com

- Posters submitted by academic institutions and industry will not be charged a fee
- Posters submitted by service providers/vendors are welcome and will be subject to evaluation by the scientific advisory board. Upon approval a fee of £399 + 21% VAT will apply

Poster application deadline: Monday 16 November 2015

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Genome Editing Applications

3 CONFERENCES - 1 LOCATION

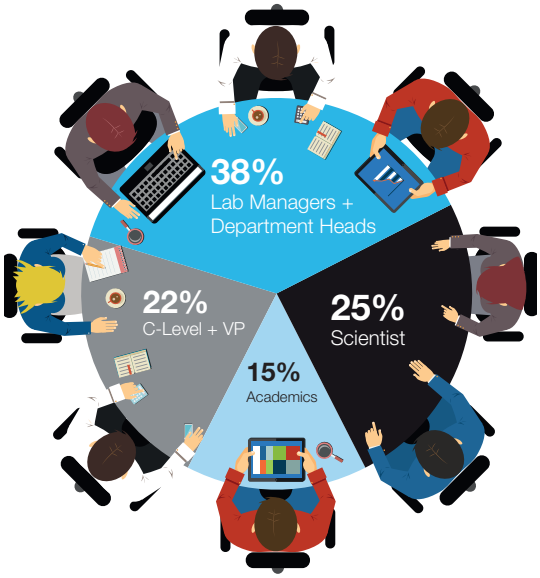
Genome Editing Applications

Cell Therapy Manufacturing

Gene Therapy

2 - 3 December 2015 - Sheraton Airport Hotel, Belgium

Primary Audience Breakdown



Vital Statistics

280+ Attendees

170+ Companies represented

62:38 buyer vs seller attendee ratio

Attendee Job Title Breakdown

CEO • CSO • Associate Director • Director • Director Functional Genomics • Director, Genetics • Head of Genome Editing • Head of Scientific Research • Manager • Professor • Scientist • Senior Director • Senior Scientist • Vice President • R&D Director

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2 -3 December 2015
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