OXFORD GLOBAL

3rd Annual Genome Editing Congress

9 - 10 November 2017, London, UK

Day 1 – Genome Editing Technologies & Techniques

- Advancements in genome editing tools:
  - CRISPR-Cas system
  - TALENs
  - Novel technologies; RNAis, ZFNs
- Gene delivery systems: viral and non-viral
- Delivery of different modifications: knockout, knockin
- Utilising genome editing in drug delivery & development
- In vivo genome editing
- Gene activation and inhibition using dead Cas9 and epigenome editing
- Updates in precise genome editing

Day 2 – Therapeutic Applications of Genome Editing

- Case studies from the areas of:
  - Oncology
  - Gene therapy
  - Inherited diseases including: cystic fibrosis; skin disease
  - Hematologic diseases
  - HIV
- Novel methods of genome editing & engineering
- Therapeutic genome editing: future challenges
- Genome editing: ethical and regulatory issues
- In vivo targeting vs. ex vivo targeting

Co-located with the **9th Annual Next Generation Sequencing Congress** & **5th Annual Single Cell Analysis Congress**

Benefits to Attending

✓ **Hear from and meet with the key innovators in genome editing.** Attendees include: VP and Senior Principal Scientist, AstraZeneca; Professor, University of Copenhagen; Professor of Genomics, Dresden University of Technology

✓ **Discover collaborative solutions to genome editing technologies and techniques.** This unique event brings together key opinion leaders to discuss advancements in CRISPR/Cas9 systems, viral and non-viral delivery systems, utilising gene editing in delivery and development

✓ **Discuss the latest innovations in the therapeutic applications of genome editing.** Case studies include haematology, oncology, inherited disease and HIV

✓ **Unparalleled networking opportunities**. The two-day congress offers dedicated networking breaks creating an interactive platform for scientific discussions. The exhibition hall and poster presentation spaces offer a relaxed and professional environment for discussion

✓ **A high-quality programme devised with the help of our esteemed advisory board.** Presentations will also cover regulatory & ethical issues and challenges in genome editing and updates in genome engineering

2017 Webinars:

- *'Genome Editing - A Tool To Transform The World: Its Promise And Some Potential Perils'.* Hosted by John Parrington, University of Oxford | Friday 8th September 2017 – Download for free
- *'A Background To Genome Editing From A Patenting Perspective'.* Hosted by Philip Webber, Dehns Patent and Trade Mark Attorneys | Friday 8th September 2017 – Download for free
- '*CRISPR Technology For Genome Editing Across Our Drug Discovery Platform'.* Hosted by Emanuela Cuomo and Marcello Maresca, AstraZeneca | Tuesday 12th September 2017 – Download for free

2017 Speakers Include:

Zoltan Ivics
Paul Ehrlich Institute

Lydia Teboul
MRC Harwell

Steven Hyde
John Radcliffe Hospital

Meet Senior Decision Makers

400 delegates from leading research & academic institutions, clinical research institutions, food & nutrition companies as well as major pharmaceutical and biotech companies will attend the event. Delegate job titles include:

Genome Editing Genome Engineering Functional Genomics	Genetics Gene Regulation Gene Therapies	Genome Biology Cell Biology Bioprocess Engineering	Biology Discovery Computational Biology Disease Modelling

Discover New Solutions

Formal and informal meeting opportunities offer delegates the chance to discuss key solutions with leading service providers. Services to be discussed include:

CRISPR TALEN ZFN	Gene Knockin Gene Knockout Detection & Analysis Tools	Gene Libraries Gene Targeting Vector Production	DNA Synthesis Bioinformatics Tools Synthetic Manufacture

For booking details & registration fees please refer to the last page or visit:
www.genomeediting-congress.com/marketing

2017 Confirmed Speakers Include:

- Rob Howes, Director, Reagents and Assay Development, Discovery Sciences, AstraZeneca
- John Feder, Associate Director, Genome Biology & Emerging Technologies, Bristol-Myers Squibb
- Laurent Poirot, Head of Early Discovery, Cellectis
- Emanuela Cuomo, Principal Scientist, AstraZeneca
- Andrea Crisanti, Professor of Molecular Parasitology, Imperial College London
- Tarik Möröy, Professor, Department of Medicine, University of Montreal
- Francis Stewart, Professor, Dresden University of Technology
- Richard Ashcroft, Professor, Queen Mary University of London
- Huw Jones, Professor, Aberystwyth University
- Ruth Chadwick, Professor, University of Manchester
- André Brändli, Professor, Ludwig-Maximilians-University Munich
- Zsuzsanna Izsvák, Professor, Max Delbrück Center for Molecular Medicine
- Pradeep Mammen, Director: Translational Research for the Advanced Heart Failure and Transplant Cardiology Program, UT Southwestern Medical Center
- Niall Barron, Director, National Institute for Cellular Biotechnology, Dublin City University
- Philip Webber, Partner, Dehns Patent and Trade Mark Attorneys
- Julia Reichelt, Head of Research, EB House
- Zoltan Ivics, Head of Division, Paul Ehrlich Institute
- Roderick Beijersbergen, Head of High Content Screening Facility, Netherlands Cancer Institute
- Lydia Teboul, Head of Molecular and Cellular Biology, MRC Harwell
- John Parrington, Associate Professor, University of Oxford
- Mark Osborn, Associate Professor, University of Minnesota
- Steven Hyde, Associate Professor of Molecular Therapy, Gene Medicine Research Group, Nuffield Division of Clinical Laboratory Sciences. John Radcliffe Hospital
- Annett Mueller, Group Leader, Division of Transfusion Medicine, Department of Haematology, University of Cambridge
- Robin Ketteler, MRC LMCB Group Leader, University College London
- Linda Popplewell, Research Officer, School of Biological Sciences, Royal Holloway University of London
- Patrick Harrison, Senior Lecturer, University College Cork
- Emmanouil Metzakopian, Career Development Fellow, Wellcome Trust Sanger Institute

2017 Confirmed Sponsor Speakers Include:

- Guilhem Tourniaire, Founder and Scientific Director, Cellenion
- Elly Sinkala, Application Scientist, cytena
- Mark Behlke, Chief Scientific Officer, Integrated DNA Technologies, Inc.
- Anja Smith, Director, Research and Development, Dharmacon
- Kevin Holden, Head of Synthetic Biology, Synthego
- Xiangyu Rao, NGS Field Application Manager, Europe, Integrated DNA Technologies
- Guillaume Pavlovic, Department Head - Genetic Engineering and Model Validation Department, Phenomin-iCS

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Congress Sponsors Continued:

3rd Annual Genome Editing Congress
Day One – 9th November 2017

07.30 – 08.20	**Registration**
08.20 – 08.25	**Oxford Global's Welcome Address**
08.25 – 08.30	**Chairperson's Opening Address**
08.30 – 09.00	**Co-Located Keynote Address:** **A Novel Validation Strategy For NGS Mutation Profiling In FFPE Tissues** We have recently developed a "Concordance Calculator" and a novel replicate approach to eliminate technical artifacts including post tissue collection modifications (PTCM) such as deamination and oxidation artifacts. Use of the Concordance Calculator to quantify reproducibility of multi-variant calls among Next Generation Sequencing replicates and to eliminate technical artifacts including PTCM also allowed us to develop an unconventional validation strategy. We call this validation approach "in situ analytical validation and evaluation (iSAVE)". This novel validation strategy and background information will be presented. **Ken Chang, Director of Clinical Biomarkers, Daiichi Sankyo**
	Genome Editing Technologies & Techniques
09.00 – 09.30	**Development And Optimisation Of CRISPR Genome Editing For Drug Discovery And Application** • New CRISPR systems, modalities and methods are being discover and published at an unprecedented pace such that unbiased and agnostic comparisons and protocol optimizations are warranted if the promise of genome engineering is to be realized in the pharmaceutical setting • We will present our results to date for generating highly optimized method for gene editing in induced pluripotent stem cells as well as examples of where and how gene editing is impacting the drug discovery process **John Feder, Associate Director, Genome Biology & Emerging Technologies, Bristol-Myers Squibb**
09.30 – 10.00	**High Fidelity Genome Editing Using RNP Complexes With A Novel Mutant HiFi Cas9** • Use of recombinant Cas9 protein as a Ribonucleoprotein (RNP) complex gives lower off-target effects (OTEs) than other approaches. • Nevertheless, OTEs still can occur and are a problem for precision editing for research and medical applications. • IDT developed a new mutant HiFi Cas9 that retains high on-target activity when used in RNP methods that dramatically reduces OTEs **Mark Behlke, Chief Scientific Officer, Integrated DNA Technologies, Inc.**
10.00 – 11.20	**Morning Coffee & Refreshments, Poster Presentation Sessions, One to One Meetings x3**
11.20 – 11.50	**Update On The CRISPR Patent Wars** • There are battles in the US between Zhang and Doudna about the ownership of the basic CRISPR technology • In Europe, 7 of Zhang's granted patents are being challenged • This presentation will discuss the background to these disputes and the current status of these patent wars **Philip Webber, Partner, Dehns Patent and Trade Mark Attorneys**
11.50 – 12.20	**The Transgenic Effect On CRISPR Innovation** **Rob Howes, Director, Reagents and Assay Development, Discovery Sciences, AstraZeneca**

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	Genome Editing Technologies & Techniques
12.20 – 12.50	**Lost In translation? Replicating Human Disease With CRISPR/Cas9** • CRISPR/Cas9 genome editing open new possibilities to develop more accurate and predictive models to better understand and treat human disease. • In this speech, we will present examples of new approaches we developed using CRISPR like CRISMERE (structural variants and CNV models) or large humanization of mouse loci. • We will also discuss the impact of CRISPR/Cas9 genome editing on the cell genome and present recommendations to improve research experimental reproducibility and safety of therapeutic applications using CRISPR **Guillaume Pavlovic, Department Head - Genetic Engineering and Model Validation Department, Phenomin-iCS** charles river
12.50 – 13.50	**Lunch**
13.50 – 14.20	**Hit Validation Strategies For Synthetic Arrayed CRISPR-Cas9 Screens** • Using high-throughput chemical synthesis of guide RNAs, we have developed the first whole genome, arrayed, synthetic CRISPR RNA library • Statistical examination of phenotypic parameters from a synthetic crRNA library high-content screen reveals robust, functional knockout for multiple reagents per gene • Strategies for prioritizing hits including confirmation of phenotype in an independent experiment, gene expression analysis, and editing efficiency will be discussed **Anja Smith, Director, Research and Development, Dharmacon** Dharmacon™ A Horizon Discovery Group Company
14.20 – 14.50	**Large Scale Combinatorial CRISPR Screens For Identification Of Genotype Specific Drug Targets** • Large scale functional genomic screens • Development clinical relevant models • Synthetic lethality • Novel drug combinations based on synthetic lethality **Roderick Beijersbergen, Head of High Content Screening Facility, Netherlands Cancer Institute**
14.50 – 15.10	**Synthetic sgRNA Enables Highly Efficient And Consistent CRISPR Editing Of Cells For Automation And Therapeutic Applications** Ribonucleoprotein (RNP) complexes between Cas9 nuclease and sgRNAs yield the highest CRISPR/Cas9 editing efficiencies with the lowest levels of off-target effects. However, consistent editing rates can be challenging for high throughput automation and therapeutic CRISPR applications. Here we demonstrate that chemically synthesized sgRNA can produce consistent genome editing efficiencies that are superior to two-piece crRNA:tracrRNA complexes and act as a more consistent replacement for in vitro transcribed guides. Furthermore, chemical modification of 5' and 3' terminal sgRNA residues with 2'-O-methyl and 3' phosphorothioate internucleotide linkages are shown to provide significant improvements to editing efficiency in primary cells. Validation of gene knockout, homology-directed repair (HDR) and ex vivo editing are demonstrated in cell lines and primary stem and T-cells. **Kevin Holden, Head of Synthetic Biology, Synthego** SYNTHEGO
15.10 – 15.30	**Cyagen - World Leader In Novel Technologies For The Rapid Generation Of Custom-Designed Animal Models And Vectors** • CRISPR/Cas9 mediated mouse/rat knockouts and knockins: point mutations, Rosa26 and any locus large fragment KI (Up to 6Kb) • TurboKnockout®, the fastest ES cell targeting platform for generation of cKO, cKI and humanized mice • PiggyBac based transgenic mice and rats • VectorBuilder.com - the only fully featured online vector tool for vector design, virus packaging, ordering, and cloning services **Matthew Wheeler, Associate Director, Cyagen Biosciences** cyagen VectorBuilder
15.30 – 16.30	**Afternoon Coffee & Refreshments, Poster Presentation Sessions, One to One Meetings x2**

3rd Annual Genome Editing Congress
Day One – 9th November 2017

	Genome Editing Technologies & Techniques
16.30 – 17.00	**Multiplex Genome Edited T-Cell Manufacturing Platform For "Off-The-Shelf" Adoptive T-Cell Immunotherapies** **Laurent Poirot, Head of Early Discovery, Cellectis**
17.00 – 17.30	**Using Recombineering To Extend The Power Of CRISPR/Cas9** • Achieving complex tasks such as humanizations, regional exchanges and conditional loxP alleles • The utility of BAC transgenes for reporters and lineage tracing, especially in iPSC models • Rapid generation of isogenic targeting constructs **Francis Stewart, Professor, Dresden University of Technology**
17.30 – 18.00	**Targeting miRNA Expression To Improve Biopharmaceutical Production In Chinese Hamster Ovary Cells** **Niall Barron, Director, National Institute for Cellular Biotechnology, Dublin City University**

18.00 – 18.30	**Therapeutic Cell Genome Editing**
	• To be discussed:
	  ○ Therapeutic cell engineering encompassing gene knockout and repair
	  ○ Off target mapping of programmable nucleases
	  ○ Cellular reprogramming/engineering
	
	Mark Osborn, Associate Professor, University of Minnesota

18.30 – 19.00	**Scaling Up - Genome Wide CRSIPR Cas9 Screening** • Introduction to CRISPR • CRISPR technology coupled with transposons • Gain and loss of function CRISPR screens in various models • CRISPR arrayed libraries generated at the Sanger institute • Summary and finishing remarks **Emmanouil Metzakopian, Career Development Fellow, Wellcome Trust Sanger Institute**
19.00 – 19.30	**Driving Genome Editing For The Development Of Malaria Vector Control** **Andrea Crisanti, Professor of Molecular Parasitology, Imperial College London**
19.30 – 20.00	**Genome Editing – A New Tool For Studying The Molecular Mechanisms Underlying Reproduction** • CRISPR/Cas9 genome editing offers a rapid and economic way to generate knockout and knockin mice • We have used this approach to study the role of sperm protein PLCzeta during fertilization in mammals • Our studies of PLCzeta knockout and knockin mice show that PLCzeta is the physiological trigger of mammalian embryogenesis **John Parrington, Professor, University of Oxford**
20.00 – 20.30	**Applying The CRISPR/Cas9 System To High Throughput Generation Of Mouse Mutants** • Utlize the CRISPR/Cas9 system in vivo at scale • Broaden the range of possible alterations • Create and analyse a library of mouse mutants to understand gene function **Lydia Teboul, Head of Molecular and Cellular Biology, MRC Harwell**
20.30 – 21.00	**Application Of Precise Genome Editing In Drug Development** • Application of CRISPR to the generation of cellular models for oncology • Application of CRISPR to the study of drug resistance **Emanuela Cuomo, Principal Scientist, AstraZeneca**
21.00 – 21.30	**Uses Of CRISPR/Cas9 Genome Editing To Study Gene Function In Autophagy** • Present our experience in using CRISPR/Cas9 genome editing to generate knockout cell lines for genes in autophagy and signaling; • Data on the use of CRISPR/Cas9 for endogenous gene tagging; • Preliminary data using CRISPR/Cas9 in systematic screening approaches; **Robin Ketteler, MRC LMCB Group Leader, University College London**
21.30	**Networking Drinks** **End of Day One**

3rd Annual Genome Editing Congress
Day Two – 10th November 2017

	3rd Annual Genome Editing Congress
	Therapeutic Applications Of Genome Editing
08.20 – 08.50	**Keynote Address:** **Ethical Issues Around Genome Editing** **Richard Ashcroft, Professor, Queen Mary University of London**
08.50 – 09.20	**Viral And Non-viral Gene Therapy For Cystic Fibrosis** • Cystic Fibrosis is the most common life threatening genetic disease in Europe and North America • The UK CF Gene Therapy Consortium has developed viral and non-viral gene transfer agents that efficiently deliver transgenes to the lungs • In pre-clinical and Phase I/IIa clinical studies in CF subjects, we have demonstrated safe, long lasting CFTR expression • In Phase IIb clinical trials we have demonstrated a halt in the progression of CF lung disease after gene delivery **Steven Hyde, Associate Professor of Molecular Therapy, Gene Medicine Research Group, Nuffield Division of Clinical Laboratory Sciences. John Radcliffe Hospital**
09.20 – 09.50	**Solution Provider Presentation** TaKaRa Clontech TaKaRa cellartis
09.50 – 10.20	**Solution Provider Presentation** **For sponsorship opportunities please contact** **sponsorship@oxfordglobal.co.uk**
10.20 – 11.00	**Morning Coffee & Refreshments, Poster Presentation Sessions, One to One Meetings x2**
11.00 – 11.30	**Superexon Correction Of Multiple CF-Causing Variants By CRISPR-mediated Homology-independent Targeted Integration (HITI)** • There are at least 272 different CF-causing variants • Superexon correction by HDR has established proof-of-principle but efficiency of repair is very low • Use of the CRISPR-HITI strategy to correct multiple CF-causing variants will be described **Patrick Harrison, Senior Lecturer, University College Cork**
11.30 – 11.50	**Technology Spotlight Presentation** **For sponsorship opportunities please contact** **sponsorship@oxfordglobal.co.uk**
11.50 – 12.10	**Technology Spotlight Presentation** **For sponsorship opportunities please contact** **sponsorship@oxfordglobal.co.uk**
12.10 – 12.40	**Ex vivo Gene Therapies For The Blistering Skin Disorder Epidermolysis Bullosa Using TALEN And CRISPR Technology** • Targeting dominant-negative mutations in EB simplex (KRT14): delete or repair? • Homology-directed repair of recessive dystrophic EB (COL7A1) using CRISPR double nicking • Off-target site analyses • Comparison of TALEN and CRISPR **Julia Reichelt, Head of Research, EB House**
12.40 – 13.30	**Lunch**
	Therapeutic Applications Of Genome Editing
13.30 – 14.00	**Transposons For Knocking Genes In And Out In Human Cells** • Advance of Sleeping Beauty transposition to the clinics • Genetic screens with transposon tools for cancer gene discovery • Next generation transposases **Zoltan Ivics, Head of Division, Paul Ehrlich Institute**

	Therapeutic Applications Of Genome Editing
14.00 – 14.30	**Development Of Gene Editing As A Therapy For Duchenne Muscular dystrophy (DMD)** • Use of various endonucleases to target mutation hotspots in the DMD gene • Correction of the mutated genetic reading frame through InDel disruption of splice acceptor sites • Homology-directed repair of the mutated DMD gene using cDNA templates • Targeting of the AAVS1 safe harbour site for microdystrophin cDNA knock-in • Optimisation of ex vivo application and description of in vivo studies in preclinical models • Use of CRISPi and CRISPa to target skeletal muscle fibrosis **Linda Popplewell, Research Officer, School of Biological Sciences, Royal Holloway University of London**
14.30 – 15.00	**Using Of Genome Editing In Mice To Understand The Role Of Gfi1 And Gfi1b In Leukemia And Non-Malignant Hematopoietic Diseases** • GFI1 and GFI1b are zing finger/SNAG domain transcription factors • GFI1 expression level and GFI1 variants are linked to development of AML and progression of MDS to AML • Hereditary mutations in both GFI1 and GFIB1 genes are linked to hematopoietic diseases such as neutropenia and bleeding disorders • Modelling of GFI1B mutations in mice using classical gene targeting and Crispr/1Cas technology reveals effect of GFI1B on platelet production **Tarik Möröy, Professor, Department of Medicine, University of Montreal**
15.00 – 15.30	**Afternoon Refreshments, Poster Presentation Sessions**
15.30 – 16.00	**The Regulation Of Genome Editing For Contained Use, Human Therapeutics And Agriculture** • Appropriate regulatory oversight will differ between contained uses, human therapeutics (both research and clinical) and agriculture • Regulatory frameworks are evolving at different rates and in different directions around the world and the current lack of clarity is already stifling innovation in some sectors • The cost and timescales of regulation will determine whether (and in what countries) this technology is adopted and commercialized **Huw Jones, Professor, Aberystwyth University**
16.00 – 16.30	**Novel Approaches to the Treatment of Duchenne Muscular Dystrophy** • Overview of Duchenne muscular dystrophy (DMD) as a neuromuscular genetic disorder • Role of genome editing as a potential treatment approach to DMD. • Potential pitfalls in utilizing genome editing as a therapeutic modality in treating DMD **Pradeep Mammen, Director: Translational Research for the Advanced Heart Failure and Transplant Cardiology Program, UT Southwestern Medical Center**
16.30 – 17.00	**Gene Editing: What Ethical Principles Apply?** In the event of the emergence of a new (bio)technology the first question to be asked is 'What's new about this?' in the case of gene editing, the comparison and contrast is undertaken vis-à-vis gene therapy. The next question is, what ethical principles should apply? This presentation will examine both these questions with relevance to obligations to future generations, autonomy and solidarity **Ruth Chadwick, Professor, University of Manchester**
17.00 – 17.30	**Genomic Engineering By Transposable Elements In Vertebrates** **Zsuzsanna Izsvák, Professor, Max Delbrück Center for Molecular Medicine**
17.30 – 18.00	**Engineering Xenopus Models Of Rare Inherited Diseases For In Vivo Drug Discovery** • Rational for developing disease models in the Xenopus model system • Utilizing gene knockdown and CRISPR/Cas9 methodologies in Xenopus • Examples of Xenopus models of rare hereditary diseases **André Brändli, Professor, Ludwig-Maximilians-University Munich**
18.00 – 18.30	**Genome Editing Approaches To Produce Universal Platelets With Added Benefits In Vitro For Clinical Applications** • Generation of an inducible hPSC lines via Zinc finger nucleases for clinical translation • Deletion of HLA-ko using CRISPR/Cas9 to produce "universal" platelets • Adding benefits to platelet by targeting candidate proteins to their alpha granules using TALEN mediated genome editing **Annett Mueller, Group Leader, Division of Transfusion Medicine, Department of Haematology, University of Cambridge**
18.30	**End of Conference**