OXFORD GLOBAL

Genome Editing Congress

12 - 13 November 2015, London, UK

Day 1 – Genome Editing Techniques

- CRISPR Cas9 system
- Zinc-finger nucleases
- TALENs
- Delivery systems: viral, nucleic acid, protein
- Achieving editing and control at whole-genome scale
- Improving genome targeting precision
- Delivery of different modifications: knockdown, knockout, knockin

Day 2 – Therapeutic Applications of Genome Editing:

- Therapeutic application case studies:
  - Cardiovascular diseases
  - Diabetes
  - Genetic disorders
  - Cystic Fibrosis
  - Gene therapy
  - Dystrophin

Co-located with our **7th Annual Next Generation Sequencing Congress & 3rd Annual Single Cell Congress**

Benefits to Attending

✓ **Hear from and meet with the key innovators in genome editing.** Attendees include: Professor, ICAN, Institute of Cardiometabolism & Nutrition; Associate Director, HTS, Antibody Discovery and Protein Engineering, MedImmune; Associate Professor, Department of Biomedicine, Aarhus University

✓ **Discover collaborative solutions to genome editing challenges.** Experts will discuss their latest and most exciting work on the application of genome editing in therapeutic areas such as cardiovascular diseases, diabetes, gene therapy and genetic disorders

✓ **Discuss the latest innovations in genome editing techniques,** including CRISPR, ZFNs, TALENs. Presentations will cover a comparison of delivery systems, genome editing modifications, improving genome targeting precision and target identification

✓ **Unparalleled networking opportunities.** The two-day congress offers ample networking opportunities creating an interactive platform for high-level scientific and business discussions. Participate in formal or informal discussions during our networking breaks and pre-organised 1-2-1 meetings

2015 Genome Editing Speakers Include…

Rafael Yáñez-Muñoz
Royal Holloway, University of London

Jean-Sebastien Hulot
Institute of Cardiometabolism & Nutrition

Myung Shin
Merck & Co., Inc

Meet Senior Decision Makers

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

Genome Editing	Molecular Biology	Biomedical Engineering	Genome Engineering
Genetics	Cell Biology	Gene Therapies	Functional Genomics
Genomics	Genetic Engineering	Gene Regulation	Computational Biology

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:

Recombination vectors	Gene design	Point mutations	Plasmid-based techniques
CRISPR	Gene replacement	Gene synthesis	Multi-locus engineering
ZFNs	Gene knockout	Gene correction	Vector design

Venue: The Genome Editing Congress will take place at will take place on the 12th & 13th November 2015, at the Novotel London West, UK.

www.genomeediting-congress.com

2015 Genome Editing Congress Confirmed Speakers:

- Jean-Sebastien Hulot, Professor, Institute of Cardiometabolism and Nutrition ICAN
- Fulvio Mavilio, Scientific Director, Genethon
- Rob Howes, Associate Director, Biologics Profiling, MedImmune
- Jacob Giehm Mikkelsen, Professor MSO, Aarhus University
- Manuel Goncalves, Associate Professor, Leiden University Medical Center
- Eric Paul Bennett, Associate Professor, Department of Odontology & Copenhagen Center for Glycomics, University of Copenhagen
- Myung Shin, Senior Principal Scientist, Merck & Co., Inc
- Marcello Maresca, Associate Principal Scientist, AstraZeneca
- Rafael J. Yáñez-Muñoz, Reader in Advanced Therapy, Royal Holloway, University of London
- Patrick Harrison, Senior Lecturer, University College Cork
- Tom Webb, Lecturer in Cardiovascular Genomics, University of Leicester
- Angelo Lombardo, Project Leader and Assistant Professor, Telethon Institute for Gene Therapy (TIGET) and Vita-Salute San Raffaele University, Milan, Italy
- Ben Davies, Head of Transgenic Core Research Group, University of Oxford
- Linda Popplewell, Lecturer, Royal Holloway University of London
- Richard Gabriel, Project Leader Targeted Genomics, National Center for Tumor Diseases, Heidelberg
- Claudio Mussolino, Junior Group Leader, Institute for Cell and Gene Therapy & Center for Chronic Immunodeficiency
- Francesco Conti, Principal Research Associate, Institute of Child Health, University College London
- Carolina Elvira Cesar, Technical Sales Specialist/Technical Support Scientist, Thermo Fisher Scientific
- Peter Lindqvist, Scientific Liaison Team Manager, EMEA, Sigma-Aldrich
- Cornelia Hampe, Product Manager for Gene Editing, Takara Clontech

2015 Next Generation Sequencing London Confirmed Speakers:

- Tim Hubbard, Head of Department of Medical & Molecular Genetics, King's College London
- Mark Beggs, Chief Executive, Stratified Medicine Scotland – Innovation Centre
- Bhushan Bonde, Associate Director, Data Integration Lead, New Medicine IT, UCB
- Simon Patton, Director, EMQN
- Darren Monckton, Professor of Human Genetics, University of Glasgow
- Jacqueline Boultwood, Professor, University of Oxford
- Massimiliano Di Ventra, Professor, UC San Diego
- Juan Pablo Couso, Professor of Developmental Genetics, University of Sussex
- Andrzej Kierzek, Professor of Systems Biology, University of Surrey
- Jan Komorowski, Professor, Uppsala University and Polish Academy of Sciences
- Jonathan Strefford, Professor of Molecular Oncology, University of Southampton
- Jean-Baptiste Cazier, Director of the Centre for Computational Biology, University of Birmingham
- Shamima Rahman, Professor of Paediatric Metabolic Medicine, UCL Institute of Child Health
- Cath Arnold, Genomic Services and Development Head, National Infection Service, Public Health England
- Surinder Singh Sahota, Reader in Immunogenetics, University of Southampton
- Peter Wild, Professor of Systems Pathology, University Hospital Zurich
- Doron Lancet, Professor, Weizmann Institute of Science
- Tony Papenfuss, Head, Computational Biology, Walter and Eliza Hall Institute of Medical Research / Peter MacCallum Cancer Centre
- Christopher Woelk, Associate Professor, University of Southampton
- Francesca Ciccarelli, Associate Professor of Cancer Genomics, Division of Cancer Studies, King's College London
- Simon Rasmussen, Associate Professor, Technical University of Denmark
- Sterghios A. Moschos, Reader in Industrial Biotechnology and Biochemistry, University of Westminster
- Mathew Upton, Reader in Medical Microbiology, Plymouth University
- Victoria Chalker, Head Respiratory and Systemic Bacteria Section, Public Health England
- Erik Sistermans, Head of Genome Diagnostics, VU University Medical Center
- Sol Efroni, Head, The Systems Biomedicine Lab, Bar-Ilan University
- Pontus Larsson, Group Leader, SNP & SEQ Technology Platform, National Genomics Infrastructure, Sweden
- Lars Grøntved, Assistant Professor, University of Southern Denmark
- Mario Capasso, Assistant Professor, University of Naples Federico II
- Jeroen de Ridder, Assistant Professor, Delft Bioinformatics Lab - Delft University of Technology
- Ana Alfirevic, Senior Lecturer, University of Liverpool
- Victor Turcanu, Senior Lecturer in Allergy, King's College London
- Justin Pachebat, Senior Lecturer Microbial Genomics, IBERS, Aberystwyth University
- Siemon Ng, Scientist, Sanofi Pasteur
- Zoltan Maroti, Senior Research Fellow, Genetic Diagnostic Laboratory, Departmet of Pediatrics, University of Szeged

2015 Single Cell Analysis London Confirmed Speakers:

- David Bensimon, Director of Research, CNRS and Professor UCLA, ENS-LPS
- Paresh Vyas, Professor of Haematology, University of Oxford
- Mathias Uhlen, Professor, KTH Royal Institute of Technology
- Jin Jen, Co-Director, Mayo Clinic
- Susann Müller, Professor, Helmholtz Centre for Environmental Research
- Yaron Shav-Tal, Professor, Bar-Ilan University
- Wei Huang, Associate Professor, University of Oxford
- Rodolphe Marie, Associate Professor, DTU Nanotech
- Anders Ståhlberg, Associate Professor, Gothenburg University
- Oscar Ces, Reader, Imperial College London
- Stephan Lorenz, Senior Scientific Manager, Wellcome Trust Sanger Institute
- Tim Roloff, Head Functional Genomics, Friedrich Miescher Institute for Biomedical Research
- Alka Saxena, Head of Genomics Core Facility, NIHR Biomedical Research Center, Guy's and St Thomas' NHS Foundation Trust
- Stefan Kirsch, Group Leader Biomarker and Technology Development, Fraunhofer ITEM-R

2015 Single Cell Analysis London Confirmed Speakers Continued:

- Pamela Pinzani, Associate Professor, University of Florence
- Gavin D. M. Jeffries, Assistant Professor, Chalmers University of Technology
- Hendrik Marks, Assistant Professor, Radboud University Nijmegen

Genome Editing, Next Generation Sequencing & Single Cell Congress Sponsors 2015:

Genome Editing Congress
Day 1 – 12th November 2015

07.30 – 08.20	**Registration: Champagne Foyer**
	Conference Room: Morangis
08.20 – 08.25	**Oxford Global's Welcome Address**
08.25 – 08.30	**Chairperson's Opening Address: Wieland Keilholz, Field Automation Specialist, NuGen Technologies**
08.30 – 09.00	**Co-located Event Keynote Address:** **The 100,000 Genomes Project** • Genomics England and the organisation of 100,000 Genomes Project • How diagnostic results will be generated and fed back to clinicians and patients • How genome and clinical data will be used enable scientific discovery and medical insight Implications for UK health system and UK economy **Tim Hubbard, Head of Department of Medical & Molecular Genetics, King's College London**

Genome Editing Congress
Day 1 – 12th November 2015

	Conference Room: Bourg
	Genome Editing Techniques
	Stream Chair: Peter Lindqvist, Scientific Liaison Team Manager, EMEA, Sigma-Aldrich
09.00 – 09.30	**ObLiGaRe: Highly Efficient Targeted Integration By Non Homologous End Joining (NHEJ) Using ZFNs, TaleNs And Cas9/CRISPR** • Application of genome editing in a large PHARMA. • ObLiGaRe allows targeted integration of large constructs even if homologous recombination is impaired • Targeted integration can be exploited to find modulator of DNA repair **Marcello Maresca, Associate Principal Scientist, AstraZeneca**
09.30 – 10.00	Delegates are welcome to attend the co-located presentations
10.00 – 10.30	**Engineered Viruses As Genome Editing Devices** • Viral vectors as delivery vehicles for genome editing tools • The impact of donor DNA structure on genome editing precision • Tailoring of genome editing strategies to target genes **Manuel Goncalves, Associate Professor, Leiden University Medical Center**
10.30 – 11.30	**Exhibition Room: Mancy & Avize** **Coffee & Refreshments, One to One Meetings x3, Poster Presentation Sessions**
11.30 – 12.00	**Targeted Genome Editing Using CRISPR/Cas9** • Design, Efficiency & Specificity of CRISPRs • CRISPR Workflow • Practical Considerations for successful Cell Line Engineering **Imran Tahir, Scientific Liaison Specialist, Sigma-Aldrich** SIGMA-ALDRICH®
12.00 – 12.30	**Genome Editing And Site-Directed Gene Insertion By Lentiviral Protein Delivery** • Aspects related to cellular delivery of genome repair kits • Concepts for incorporating nucleases in lentivirus-derived particles • Data showing co-delivery of nucleases and repair donor in viral particles • Examples of site-directed gene insertion by viral protein delivery in stem cells **Jacob Giehm Mikkelsen, Professor MSO, Aarhus University**
12.30 – 13.00	**Genome Editing Solutions – New Workflow Tools For Genome Editing Genome Editing Workflow** • Design, Detection and Genome Editing tools using CRISPR • 4 days from design to results • High Efficiency editing in difficult cell lines using cas9 protein • Improved delivery using Neon electroporation or CRISPRMAX **Carolina Elvira Cesar, Technical Sales Specialist/Technical Support Scientist, Thermo Fisher Scientific** ThermoFisher SCIENTIFIC
13.00 – 14.00	**Exhibition Room: Mancy & Avize** **Lunch**
14.00 – 14.30	Delegates are welcome to attend the co-located presentations
14.30 – 15.00	**Novel Improved Targeting And Indel Identification Methodologies For Genome Editing** The emerging Gene editing tools for precise engineering of higher eukaryote genomes such as ZFNs, TALENs, CRISPR/Cas or Meganucleases, have revolutionized bioscience. In contrast to the speed by which these editing tools are being optimized and strategies for high throughput use in whole-genome screens are devised, considerable less focus are being devoted to improving capabilities for targeting, detection and characterization of the induced insertions and/or deletions (indels) at the specific breakpoint as well as at potential off-targets. Here, we report a novel strategy, coined IDAA for Indel Detection by Amplicon Analysis, that combines use of a simple amplicon labelling strategy with the high throughput capability of DNA fragment analysis by automated Capillary Electrophoresis for simple detection and characterization of indels induced by precise gene targeting. This presentation will also cover the description of a versatile method for increasing CRISPR/Cas9, ZFN as well as TALEN editing efficiencies by co-expression of nuclease and fluorescent protein in the same cell, which translates into increasingly higher genome editing frequencies. **Eric Paul Bennett, Associate Professor, Department of Odontology & Copenhagen Center for Glycomics, University of Copenhagen**

Genome Editing Congress
Day 1 – 12th November 2015

	Conference Room: Bourg
15.00 – 15.30	**Permanent Epigenetic Silencing Of Human Genes With Artificial Transcriptional Repressors** • Repressive epigenetic states deposed during development on endogenous retroviruses can be permanently propagated throughout adult life • Development of Artificial Transcriptional Repressors (ATRs) based on the molecular machinery involved in the silencing of endogenous retroviruses • Exploitation of the ATRs to silence endogenous genes of therapeutic relevance **Angelo Lombardo, Project Leader and Assistant Professor, Telethon Institute for Gene Therapy (TIGET) and Vita-Salute San Raffaele University, Milan, Italy**
15.30 – 16.00	Delegates are welcome to attend the co-located presentations
16.00 – 17.00	**Exhibition Room: Mancy & Avize** **Afternoon Refreshments, One to One Meetings x3, Poster Presentation Sessions**
17.00 – 17.30	**Genome Editing To Generate Coronary Artery Disease Cell Models** • Genome editing to investigate complex genetics • Homologous recombination to target coding and non-coding disease associated variants • Engineered promoters and reporters to investigate gene regulation and expression **Tom Webb, Lecturer in Cardiovascular Genomics, University of Leicester**
17.30 – 18.00	**Genome-wide Analyses Of Gene Editing – On-and Off-target Modifications** • Current techniques to analyse off-target activity • Off-target activity of different (types of)designer nucleases • Quantifying on-target modifications **Richard Gabriel, Project Leader Targeted Genomics, National Center for Tumor Diseases, Heidelberg**
18.00 – 18.30	**Application Of CRISPR/Cas9 For The Generation Of Genetically Modified Mouse Models** • Generation of a mouse model overexpressing Cas9 • High rates of mutagenesis achieved in oocytes from Cas9 overexpressing mice **Ben Davies, Head of Transgenic Core Research Group, University of Oxford**
18.30	**Exhibition Room: Mancy & Avize** **Networking Drinks: End of Day One**

Genome Editing Congress
Day 2 – 13th November 2015

	Conference Room: Bourg
	Therapeutic Applications Of Genome Editing
	Stream Chair: Myung Shin, Senior Principal Scientist, Merck & Co., Inc
08.30 – 09.00	**Genome Editing For Blood Genetic Diseases** • Gene replacement in hematopoietic stem cells showed remarkable efficacy as a treatment for severe and incurable blood genetic diseases; however, non-controlled integration of gene transfer vectors still poses issues of biosafety and long-term genotoxicity • Precise editing of genes and regulatory elements may provide a safer and efficacious alternative to the use of viral vectors for genetic modification of stem cells • CRISPR/Cas9 technology offers a flexible platform for genetic modification of hematopoietic stem cells: gene knock-out, deletion of regulatory elements and eventually gene correction may be addressed by this technology by either viral or non-viral delivery to stem cells ex vivo **Fulvio Mavilio, Scientific Director, Genethon**
09.00 – 09.30	Delegates are welcome to attend the co-located presentations
09.30 – 10.00	Delegates are welcome to attend the co-located presentations
10.00 – 10.30	**Exhibition Room: Mancy & Avize** **Coffee & Poster Presentations, One to One Meetings x1, Poster Presentation Sessions**
10.30 – 11.00	**Genome Editing Of Human iPS Cells To Study And Correct Inherited Cardiac Disorders** • Use of genome-editing techniques to target missense mutations causing genetic cardiomyopathies • Use of genome-editing techniques to improve prediction of cardiotoxic effects of drugs • Current strategies with Cas9/gRNA: start with patient-specific or control iPS cells ? • Insight into CRISPR possiblity to correct inherited cardiac disorders **Jean-Sebastien Hulot, Professor, Institute of Cardiometabolism and Nutrition ICAN**
11.00 – 11.30	**Application Of Gene Targeting Technologies In Biologics Drug Discovery** • This presentation will discuss: • Introducing Biologics drug discovery and the specific challenges we face • Use of CRISPR to develop isogenic cell lines and their use in specific projects for metabolic disease **Rob Howes, Associate Director, Biologics Profiling, MedImmune**

Genome Editing Congress
Day 2 – 13th November 2015

	Conferene Room: Bourg
11.30 – 12.00	**New Tools For Successful CRISPR/Cas9 Genome Editing - Discover How To Speed Up Your Workflow With Guide-it™!** • CRISPR/Cas9 gene editing in 5 easy steps, focusing on Takara Clontech´s latest innovative tools for genome editing: - Choose your target - Design your sgRNA - Test sgRNA efficacy *in vitro* - Deliver sgRNA and Cas9 to your cells or *in vivo* - Monitor INDELS **Cornelia Hampe, Product Manager for Gene Editing, Takara Clontech, France**
12.00 – 12.30	Delegates are welcome to attend the co-located presentations
12.30 – 13.30	**Exhibition Room: Mancy & Avize** **Lunch**
	Stream Chair: Jean-Sebastien Hulot, Professor, Institute of Cardiometabolism and Nutrition ICAN
13.30 – 14.00	Delegates are welcome to attend the co-located presentations
14.00 – 14.30	**Rescue Of T-cell Deficiency In Prkdc Scid Mice By Ex Vivo Gene Editing** • ZFNs were used to repair Prkdc in scid fibroblasts and lin- HSCs in vitro • Transplanted gene-edited lin- HSCs rescued T-cell deficiency in some of the primary and secondary recipient mice • No significant off-target ZFN activity was detected in samples from the transplantation experiment **Rafael J. Yáñez-Muñoz, Reader in Advanced Therapy, Royal Holloway, University of London**
14.30 – 15.00	**CRISPR Cas9/gRNA Editing For Cystic Fibrosis** • Cas9/gRNA used to repair F508del mutation with donor plasmid • Insight into repair mechanism • Use of Cas9/gRNA to target class I splice mutations • Use of Cas9/gRNA to target class I premature stop codon mutations **Patrick Harrison, Senior Lecturer, University College Cork**
15.00 – 15.30	**Genome Editing Tools To Tackle HIV Infection** • Overview of different genome editing tools • Genome editing for HIV therapy • Specificity of designer nucleases, ZFNs, TALENs, CRISPRs **Claudio Mussolino, Junior Group Leader, Institute for Cell and Gene Therapy & Center for Chronic Immunodeficiency**
15.30 – 16.00	**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 ssODN and cDNA templates • Optimisation of ex vivo application and description of in vivo studies in preclinical models **Linda Popplewell, Lecturer, Royal Holloway University of London**
16.00 – 16.30	**Exhibition Room: Mancy & Avize** **Afternoon Refreshments, Poster Presentation Sessions**
16.30 – 17.00	**Application Of Genome Editing In Animal Models For Drug Discovery** • Use of Genome Editing tools to generate genetically engineered models beyond mice • Use of CRISPR rapid generation of mouse models • Use of Genome Editing tools to make human genetic mutation in preclinical models **Myung Shin, Senior Principal Scientist, Merck & Co., Inc**
17.00 – 17.30	**Correction Of Mutations In Dystrophin Using CRISPR/Cas9 Nucleases** • 10-15% of patients with Duchenne Muscular Dystrophy have mutations in one or more exons of the dystrophin gene • These mutations are difficult to address with current approaches • We aim to correct large duplications using a single CRISPR/Cas9 nuclease **Francesco Conti, Principal Research Associate, Institute of Child Health, University College London**
17.30 – 18.00	Delegates are welcome to attend the co-located presentations
18.00	**End of Congress**