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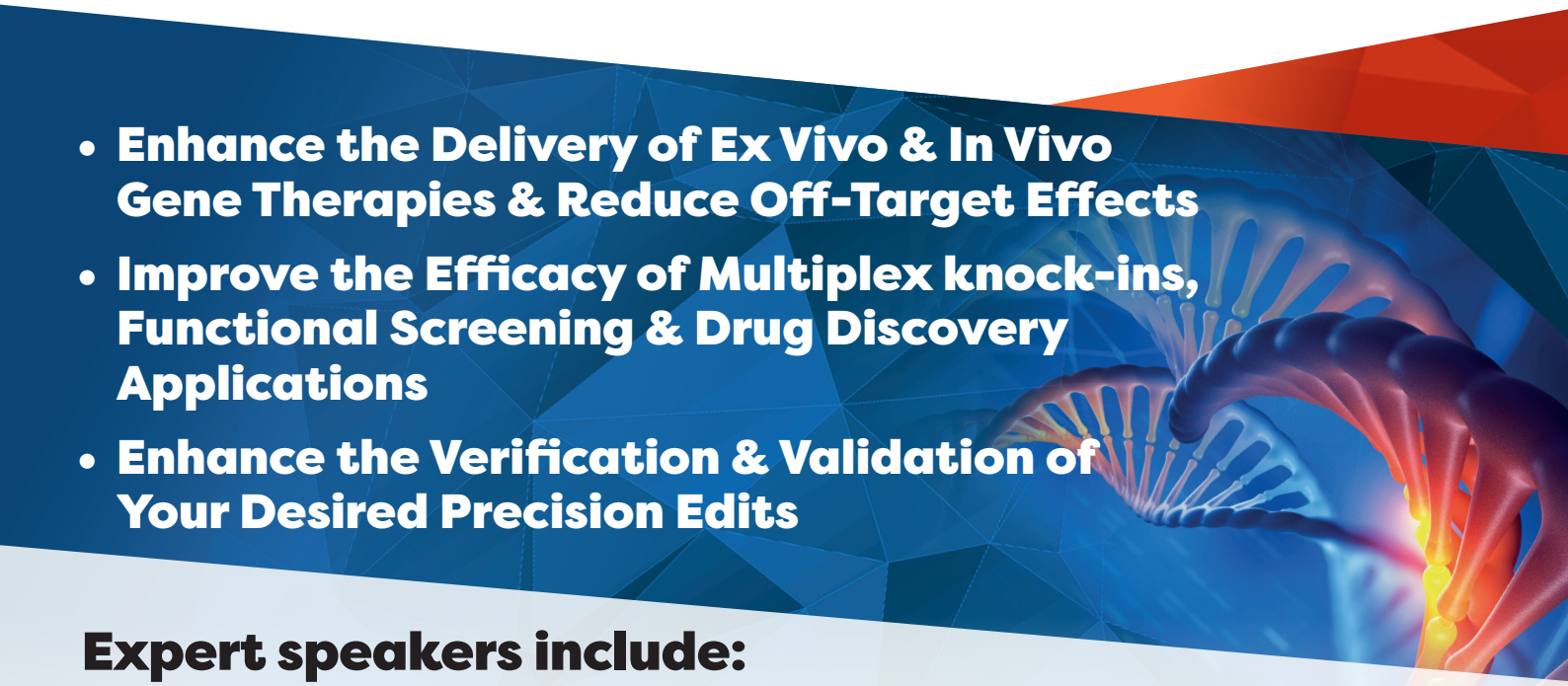
PRECISION CRISPR

DRUG DISCOVERY & GENE THERAPY EUROPE 2018

- **Enhance the Delivery of Ex Vivo & In Vivo Gene Therapies & Reduce Off-Target Effects**
- **Improve the Efficacy of Multiplex knock-ins, Functional Screening & Drug Discovery Applications**
- **Enhance the Verification & Validation of Your Desired Precision Edits**



Expert speakers include:

Jacob Corn
Professor
ETH Zürich

Fredrik Wermeling
Assistant professor
Karolinska Institue

Eric Paul Bennet
Associate professor
University of Copenhagen

Andrew Bassett
Head of Research (Cellular Operations)
Wellcome Trust Sanger Institute

Lin Wu
Director
Genome Modification Facility

Barry Rosen
VP & Senior Principal Scientist
AstraZeneca

Roberto Nitsch
Associate Director,Advanced Medicines Safety group
AstraZeneca

Channabasavaiah Gurumurthy
Director
UNMC

Danilo Maddalo
Head of Lab
Novartis

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Welcome to the 3rd Precision CRISPR: Drug Discovery & Gene Therapy 2018

Precision CRISPR: Drug Development & Gene Therapy Congress returns 22-24 October 2018, just in time to join the tangible excitement as CRISPR gene therapies move into the clinic. Join us to take part in the conversations around the future of CRISPR gene editing in Europe and the rest of the world.

Through a dynamic program of focused sessions, interactive discussions and networking opportunities, you will leave this meeting with a deep understanding of:

- the challenges in delivery & reducing off-target effects in ex vivo & in vivo gene therapies
- how the likes of GSK, Novartis, AstraZeneca and more, work towards improving the efficacy of functional screening and drug discovery
- the most recent gene editing techniques and Cas variants poised to make precise gene editing all that more precise

Become part of a dedicated community in Europe, striving to realise the drug discovery & gene therapy applications of optimised CRISPR systems.

"I am looking forward to get inspired by the novel CRISPR based biotechnologies and their cutting edge therapeutic applications"

Qianxin Wu, Speaker, Precision CRISPR 2018

"Exploitation of the potential for precise genome editing at the single base"

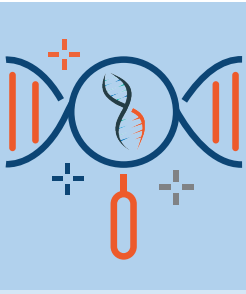
Eric Paul Bennett, Speaker, Precision CRISPR 2018

5 Key Takeaways from Precision CRISPR EU

Discover the mechanisms of HDR in human cells, the challenges of editing in hematopoietic stem cells and the progress towards translation of gene editing therapies for sickle cell disease with **Jacob Corn of ETH Zurich**

Discover how to **create designer animal models** to create translatable preclinical research with insights from Guru Channabasavaiah of UNMC

Understand how AstraZeneca utilise precise genome engineering to **empower drug discovery**



Discover how to detect in vitro and ex vivo Cellular indel Profiles and Dynamics cause by **CRISPR/Cas 9 delivery formats** with Eric Paul Bennett, University of Copenhagen

Learn how Novo Nordisk are utilizing **Single-cell CRISPR sequencing** to empower their drug discovery

EXPERT SPEAKERS

Jacob Corn
Professor of Genome Biology
ETH Zurich

Fredrik Wermeling
Assistant professor
Karolinska Institue

Eric Paul Bennet
Associate professor
University of Copenhagen

Andrew Bassett
Head of Research (Cellular Operations)
Wellcome Trust Sanger Institute

David Dow
Early Program Leader - Cell & Gene Therapy
GSK

Lin Wu
Director
Genome Modification Facility, Harvard University

Quin Wills
Department Head, Cellular and Systems Genomics
NNRCO

Silvia Ottaviani
Research Associate
Imperial College London

Channabasavaiah Gurumurthy
Director
UNMC

Ali Nowrouzi
Senior Scientist
DFKZ

Barry Rosen
VP & Senior Principal Scientist
AstraZeneca

Leandro Castellano
Honorary Senior Lecturer
Imperial College London

Qianxin Wu
Scientist
Wellcome Trust Sanger Institute

Adriana Beltran
Research Assistant Professor
UNC chapel Hill

Lorena Benedetti
Research Associate
The Francis Crick Institute

Garrett Rettig
Director of Product Development, Molecular Genetics
Integrated DNA Technologies, Inc.

Kevin Holden
Head of Synthetic Biology
Synthego

Shamim Rahman
Senior Research Scientist
Novo Nordisk

Roberto Nitsch
Associate Director,Advanced Medicines Safety group
AstraZeneca

Tony Perry
Head of the Laboratory of Mammalian Molecular Embryology
University of Bath

Asma Siari
Technology & Market Analyst
Yole Développement

Conference Day One | Tuesday, 23rd October 2018

8.00 Registration

Leandro Castellano
Honorary Senior Lecturer
Imperial College London

9.00 Chair's Opening Remarks

Slipping Under the Radar No More; Learn How to Achieve Absolute Identification & Verification of On- & Off- Target Gene Editing and HDR Events

Jacob Corn
Professor of Genome Biology
ETH Zurich

9.10 Gene Repair in Human Hematopoietic Stem Cells

- A systematic approach for generating knock-ins in stem cells
- Identifying and improving precision of HDR events
- Use of live imaging and gene editing to explore cell states

Eric Paul Bennet
Associate professor
University of Copenhagen

9.40 Detection of in vitro and ex vivo Cellular indel Profiles and Dynamics Induced By Different CRISPR/Cas9 Delivery Formats

- Methodologies for absolute identification of cellular Cas9 induced indel events
- Plasmid-, piggyback-, lenti- and RNP -Cas9 delivery formats
- Indel formation dynamics and profiles induced by various CRISPR/Cas9 delivery methods
- In vitro and ex vivo Indel formation, dynamics and profiles induced by various CRISPR/Cas9 delivery formats

Garrett Rettig
Director of Product Development, Molecular Genetics
Integrated DNA Technologies, Inc.

10.10 Target Enrichment and High-Resolution NGS Analysis of On- and Off-target CRISPR/Cas9 Editing Events

- Multiplexed PCR with rhAmpSeqTM enables on- and off-target assessment of Cas9 editing in a single reaction.
- This technology is applied to quantification of editing events for CRISPR/Cas9 via RNP delivery as well as IDT AltR® HiFi Cas9, a novel protein that maintains on-target potency while increasing specificity.
- Orthogonal methods for detecting on-target and off-target editing are compared to predictions via available algorithms.

10.40 Speed Networking

11.00 Morning Refreshments

Genetically Modified Drug Discovery: Discover How Precise Genome Engineering can Empower Your Drug Discovery Pipeline

Barry Rosen
VP & Senior Principal Scientist
AstraZeneca

11.20 Precise Genome Engineering to Empower Drug Discovery

- Pooled and arrayed CRIPSR screening for target Identification
- Target Validation in CRISPR enabled cell and animal models of disease
- Precision medicine approaches in Oncology and beyond

Asma Siari
Technology & Market Analyst
Yole Développement

11.50 CRISPR Technology & Market Overview: challenges, market competition landscape and strategic opportunities

- CRISPR global market dynamics: collaborations, mergers and acquisitions, fundraising and strategic product developments
- Market data and forecasts over the period 2017 - 2023, and the main players for each segments (biotechnologies, agritech, therapeutics and diagnostics)
- Roadmaps with an overview of the latest innovations per each segments
- Focus on the emerging business models for CRISPR technology
- Patent landscape analysis

Roberto Nitsch
Associate Director,Advanced Medicines Safety group
AstraZeneca

12.05 CRISPR Can Make it Safe: *In Vitro, In Vivo,* In Clinic

- The simplicity of editing genomes with CRISPR engaged the scientific community to think about its application to the clinic without a thorough consideration of any potential intrinsic harmful effects.
- The major drawback of CRISPR is currently represented by its Off-Target activity, which can cause unwanted disruption of gene functions and more broadly, genomic instability which result detrimental to the fitness of the targeted cell.
- In order to move CRISPR closer to the clinics, we need to improve both efficacy and safety and broaden the spectrum of applications to more target-organs in vivo and ex vivo.

12.35 Networking Lunch

The Next Top Model: Utilise CRISPR To Create the Next Generation of Disease Relevant & Translatable Models

Channabasavaiah Gurumurthy
Director
UNMC

13.35 Creating Designer Animal Models Made Easier Using Easi-CRISPR and GONAD Technologies

- The presentation will cover latest developments in enhancing insertion of larger DNA cassettes.
- The CRISPR strategies that help rapid development of complex and much-needed animal models.

Lin Wu
Director
Genome Modification Facility

14.05 Mouse Genome Editing Using CRISPR/Cas9 Technology

- Different forms of Cas9 and gRNA used for genome editing
- Targeted gene disruption and deletion to generate gene KO mice
- Site-specific transgene integration to generate gene KI mice

Adriana Beltran
Director Human Pluripotent Stem Cell Core
UNC

14.35 CRISPRing iPSCs to Efficiently Model Genetic Diseases

- Generating iPSC from patients with genetic disorders
- Creating isogenic iPSCs for disease modeling with the CRISPR/Cas9 system
- Applying the CRISPR/Cas9 system to Cerebral Cavernous Malformation (CCM) patientspecific iPSCs to generate knockout models
- Developing 3D culture platforms of iPSCs-derived endothelial cells for transcriptome and proteomic studies

15.05 Afternoon Refreshments & Poster Session

This is a complimentary poster session and the opportunity to present is included in the standard event pass. The maximum size poster we can accommodate is A0 (33.1 x 46.8 inches), either landscape or portrait. **Please be aware this opportunity is only available to Drug Developers and Academics.**

15.45 Interactive Round Table Discussions

1. Developing Next Generation Animal & Organoid Models for the most Translatable Preclinical Research

2. Utilising Novel Delivery Methods to Overcome the Difficulties in Delivering Gene Editing Technology

3. Overcoming the Challenges of Utilising CRISPR Gene Editing as a Gene Therapy. What Challenges Persist to Prevent & What Pioneering Research Paves the Way to the Clinic?

16.55 Close of Day 1

Conference Day Two | Wednesday, 24th October 2018

8.30 Registration & Coffee

9.00 Chair's Opening Remarks

Andrew Bassett
Head of Research (Cellular Operations)
Wellcome Trust Sanger Institute

9.10 Modelling neurodegenerative disease using isogenic pairs of human iPSCs generated by CRISPR/Cas9

- Efficient production of isogenic iPSC lines using CRISPR RNP and high throughput sequencing
- Use of isogenic cell pairs to understand the causative genetic aberrations, independent of genetic background
- Identification of perturbed cellular stress responses in an iPSC-derived model of Parkinson's disease (alpha-synuclein triplication)

Silvia Ottaviani
Research Associate
Imperial College London

9.40 The role of non-coding RNAs regulated by TGF-b in controlling pancreatic cancer Progression

- We have unravelled the microRNAs controlled by TGF-b and characterised their role in the TGF-b-mediated tumourigenesis
- We have used several CRISPR-related approaches to disentangle the role of these microRNAs in cancer progression
- We have discovered that microRNAs transcribed in cluster from the same primary transcript can have opposite role in controlling tumourigenesis

Kevin Holden
Head of Synthetic Biology
Synthego

10.10 Synthetic sgRNA Enables Highly Efficient & Consistent CRISPR Editing of Primary Cells for Therapeutic Applications

- Achieving high editing efficiencies in CRISPR therapeutic applications while maintaining consistency remains a significant challenge
- Traditional methods for generating guide RNAs can yield molecules of inconsistent length and quality that affect genome editing efficiency
- We demonstrate that synthetic sgRNA produces consistent editing efficiencies superior to two-piece crRNA:tracrRNA complexes and IVT-derived guides

10.25 Morning Refreshments

Lorena Benedetti
Research Associate
The Francis Crick Institute

10.55 Identification of New Cancer Genes Allow Patient Stratification in Oesophageal Adenocarcinoma

- Oesophageal adenocarcinoma is the eighth most frequent cancer worldwide, with a five-year survival rate below 20% -Recurrence of cancer driver gene alterations is rare and available computational tools are unable to identify cancer driver genes in around 10-20% of patients
- We developed a machine learning algorithm that identifies and rank cancer-related genes in all patients
- We experimentally validated the contribution of some of these genes in cell lines by mimicking their perturbation found in cancer patients using either synthetic CRISPR for gene KO or lentiviral mediated gene overexpression

Fredrik Wermeling
Assistant professor
Karolinska Institue

11.25 Utilising CRISPR Screen in Difficult Cells, Studies of Primary Immune Cells

Qianxin Wu
Scientist
Wellcome Trust Sanger Institute

11.55 Functional Dissection of RNA Regulatory Elements with CRISPR Cas9

- Developing a genome engineering based biotechnology (GenERA) to functionally dissect the 3'UTR and RNA cis regulatory elements in their native context
- GenERA can be used to unbiasedly explore the regulatory landscape of a entire UTR
- GenERA reveals cooperativity among multiple RNA regulatory elements.
- GenERA allows functional interrogation of a microRNA target network

12.25 Networking Lunch

Ali Nowrouzi
Senior Scientist
DFKZ

13.25 Utilizing CRISPR/Cas9 Functional Screens to Understand the Dynamics of Tumor Evolution and Radiation resistance

- Clonal Evolution of Tumor recurrencs and distant metastasis
- Identifying Synthetic lethal targets in combination with Radiation and DNA repair inhibitors.
- Mechanisms of Therapy resistance
- Safety and efficacy of genome editing

Tony Perry
Head of the Laboratory of Mammalian Molecular Embryology
University of Bath

13.55 A genome Editing Switch

- How genetic code expansion works in mammalian cells
- Switching on Cas9 activity with a non-physiological amino acid, BOC
- Examples of heritable, BOC-induced genome editing
- Some potential applications

David Dow
Early Program Leader - Cell & Gene Therapy
GSK

14.25 The Impact of CRISPR on T cell Cancer Immunotherapies

- Successes and challenges in T cell cancer immunotherapy
- The potential of CRISPR to address some of the challenges
- Future perspectives

14.55 Chair's Closing Remarks

15.00 Close of the 3rd CRISPR Europe 2018

PART OF THE PRECISION CRISPR SERIES

PRECISION CRISPR STEM CELL

PRECISION CRISPR DRUG DISCOVERY & GENE THERAPY BOSTON 2018

Pre-Conference Workshop Day | Monday 22 October 2018

Harnessing The Next Generation of In Vivo Models for Basic, Drug Development & Translational Research

Workshop A

Designing Transgenic Animal Models Using the CRISPR Genome Editing System

10am - 1pm

Designing the designer animal models using CRISPR genome editing system in this workshop you will discover:

- Newer technical breakthroughs in transgenic technologies that have been possible because of the CRISPR/Cas9 system
- Designing common and advanced type of designer animal models for basic, translational and pharmaceutical research
- Case studies: evaluation of animal model designs of participants' examples

Workshop Leader
Guru Channabasavaiah
Associate Professor
UNMC

CB Gurumurthy is an Associate Professor of Developmental Neuroscience, Munroe Meyer Institute for Genetics and Rehabilitation, and he serves as the Director of UNMC Mouse Genome Engineering Core Facility at the University of Nebraska Medical Center. He received his BVSC (DVM) from Bangalore Veterinary College, India and MVSC & PHD in Veterinary Virology from Indian Veterinary Research Institute with a Gold Medal distinction in MVSC. Prior to his current position, he served as a Molecular Biology Scientist, Drug Discovery Research at Dr. Reddy's Laboratories, and then he moved to Northwestern University, Chicago for a Post-Doctoral work in Cancer Biology and Mouse Molecular Genetics. He also received his Executive MBA from University of Nebraska at Omaha. His research interests are improving and development of novel genome engineering technologies.

Workshop B

Optimising the Development and Application of CRISPR Derived Transgenic Mice Models

2pm - 5pm

Workshop Leader
Lin Wu
Director
Genome Modification Facility

Lin Wu received her Ph.D. from Columbia University in Molecular and Cellular Developmental Biology, and her Postdoctoral training from The Rockefeller University in Biomedical Genetics and Metabolic diseases. After working many years as an associate director at the Transgenic and Gene Targeting Facility of Massachusetts General Hospital, Harvard Medical School, Lin became the director of the Genome Modification Facility (GMF) at Harvard University in 2012. The GMF provides transgenic, gene targeting, and other services to investigators of Harvard University and its affiliated institutions, as well as to investigators within the US and abroad. For more details, please see http://gmf.fas.harvard.edu/

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Synthego is a leading provider of genome engineering solutions. The company's flagship product, CRISPRevolution, is a portfolio of synthetic RNA designed for CRISPR genome editing and research. Synthego's vision is to bring precision and automation to genome engineering, enabling rapid and cost-effective research with consistent results for every scientist. Headquartered in Silicon Valley, California, Synthego customers include leading institutions in 31 countries around the world, and 9 of the top 10 global biology universities.

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Exhibitor

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Exhibitor

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