



CRISPR

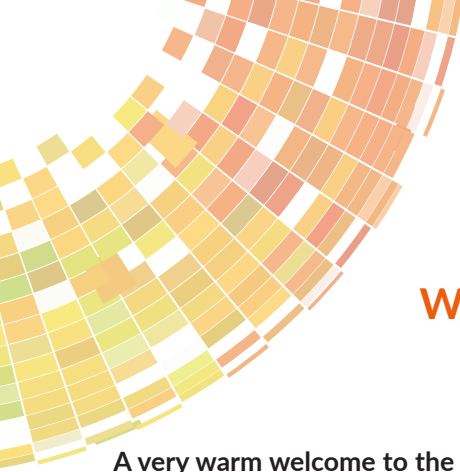
In Drug Discovery 2021

From Targets to Therapeutics

23 - 24 March 2021



www.elrig.org



Welcome to CRISPR In Drug Discovery 2021

A very warm welcome to the delegates, speakers and exhibitors joining us for our third CRISPR in Drug Discovery conference organised by ELRIG.

We are pleased to be able to host you virtually from Cambridge, a centre of academic and biotechnology excellence. The impact of CRISPR-based genome engineering technologies has been revolutionary across all phases of drug discovery since its inception as a genome editing tool eight years ago. Over the next two days, we intend to demonstrate the profound impact of CRISPR technologies on the present and the future of drug discovery through a series of presentations by world class speakers and technology providers who will illustrate both the current accomplishments and future possibilities of the rapidly evolving methodologies around CRISPR genome engineering. We are especially pleased to have a group of distinguished keynotes from Mathew Garnett, a leader in functional genomic approaches to understand cancer dependencies and one of the leads in the DepMap project, Madeline Lancaster, a pioneer in the development and use of brain organoids to understand development and disease, and Jacob Corn, an innovator in developing genome engineering approaches and their translation to the clinic.

Sessions will focus on the power of CRISPR based methods to identify novel drug targets through the application of pooled and arrayed CRISPR functional genomic screens including those with single cell 'omics readouts. This will be followed by applications in target validation in disease relevant cellular systems, including human stem cells and complex models, such as organoids.

We will conclude with a session looking ahead to the potential of CRISPR based technologies to be utilized as tools for therapeutic genome engineering of somatic cells, especially in the treatment of genetic diseases.

The podium program has been complemented by presentations from leading CRISPR reagent and service providers that will be of broad scientific interest and highlight the accessibility of such technology. The scientific program will be rounded out by a poster session and vendor exhibition, allowing participants the opportunity to engage directly with application scientists and explore the latest instrumentation and reagents offered by exhibitors.

The organisation of this meeting required hard work and dedication from many individuals. We would like to thank the ELRIG operations team; Clare Cockerham, Tara Shanks and Sanj Kumar for their administrative support and Melanie Leveridge, the chair of ELRIG for helping to develop the initial concept of this unique conference.

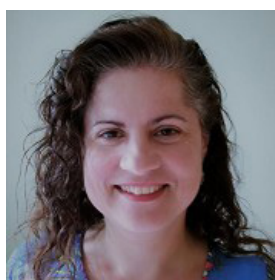
ELRIG is a not-for profit organisation whose mission is to provide outstanding, leading-edge knowledge to the life sciences community on an open access basis. This would not be possible without the generous support of the exhibitors. We are grateful to them all and especially to our platinum sponsors; Horizon and Iota Sciences. Please do take some time to engage with them during the refreshment breaks.

We hope that you all enjoy the symposium over the next two days and look forward to meeting you!

CRISPR In Drug Discovery 2021 Conference Directors



Andrew Bassett
(Wellcome Sanger Institute)



Dr Klio Maratou
(GlaxoSmithKline)



Dr Marcello Maresca
(AstraZeneca)

DAY 1: TUESDAY 23RD MARCH

CONFERENCE HALL

FUNCTIONAL GENOMICS

*All times are GMT

11:00 - 11:05	Conference Welcome & Plenary Keynote Introduction: Andrew Bassett (<i>Wellcome Sanger Institute</i>) Dr Klio Maratou (<i>GlaxoSmithKline</i>) Dr Marcello Maresca (<i>AstraZeneca</i>)
11:05 - 11:50	PLENARY KEYNOTE Functional Genomics to Guide Cancer Drug Discovery Dr Mathew Garnett (<i>Wellcome Sanger Institute</i>)
11:50 - 12:20	HORIZON DISCOVERY PLATINUM SPONSOR CRISPR screening to identify new drug targets in complex cell models Dr Carlos Le Sage
12:20 - 13:20	REFRESHMENT BREAK, POSTER SESSION AND VENDOR EXHIBITION
13:20 - 13:25	SESSION CHAIR INTRODUCTIONS Andrew Bassett (<i>Wellcome Sanger Institute</i>)
13:25 - 13:45	Using CRISPR/Cas9 screens to study epidermal keratinocyte differentiation and inflammation Dr Christopher Bryant (<i>Wellcome Sanger Institute</i>)
13:45 - 14:05	Multilayered VBC score predicts sgRNAs that efficiently generate loss-of-function alleles Ulrich Elling (<i>Institute of Molecular Biotechnology, IMBA, Vienna</i>)
14:05 - 14:25	Joint Q&A session Mathew Garnett (<i>Wellcome Sanger Institute</i>) Dr Christopher Bryant (<i>Wellcome Sanger Institute</i>) Ulrich Elling (<i>Institute of Molecular Biotechnology, IMBA, Vienna</i>)
14:25 - 14:40	ABCAM INDUSTRY INSIDER FirePlex Multiplexed Assay Technology – a Cell-line Profiling Tool Zeev Gechtman
14:40 - 15:00	REFRESHMENT BREAK
15:00 - 15:20	Screening for functional transcriptional and splicing regulatory variants with GenIE Dr Sarah Cooper (<i>Wellcome Sanger Institute</i>)
15:20 - 15:40	CRISPR/Cas9 - from functional genomics applications to therapeutic genome editing Dr Lars Steinmetz (<i>EMBL</i>)
15:40 - 15:55	SYNTHIGO INDUSTRY INSIDER CRISPR Tools to Mitigate Attrition Rate Risk in Drug Development Dr Kai Wilkens
15:55 - 16:00	10X GENOMICS PREMIUM SPONSOR Accelerate functional genomics studies with simultaneous capture of CRISPR guides and gene expression profiles at single cell resolution Stephen Hague
16:00 - 16:15	REFRESHMENT BREAK
16:15 - 16:35	Ultra-high throughput single-cell RNA sequencing and perturbation screening by combinatorial fluidic indexing Dr Paul Datlinger (<i>CeMM Research Center for Molecular Medicine, Vienna</i>)
16:35 - 16:40	AELIAN BIOTECHNOLOGY PREMIUM SPONSOR A single-cell CRISPR screen in >100k primary human T cells Dr Tilmann Buerckstueimmer
16:40 - 17:00	Advanced iPSC technologies for neurodegenerative disease research Michael Ward (<i>NIH/NINDS</i>)
17:00 - 17:20	Joint Q&A session Dr Sarah Cooper (<i>Wellcome Sanger Institute</i>) Dr Lars Steinmetz (<i>EMBL</i>) Dr Paul Datlinger (<i>CeMM Research Center for Molecular Medicine, Vienna</i>) Michael Ward (<i>NIH/NINDS</i>)
17:20 - 17:50	HORIZON DISCOVERY HOT TOPIC DISCUSSION Understanding the paradigm of cellular visualization

DAY 2: WEDNESDAY 24TH MARCH
CONFERENCE HALL
TARGET VALIDATION AND THERAPEUTICS
 SPONSORED BY **iotaSciences**

11:00 - 11:05	Conference Welcome & Plenary Keynote Introduction: Andrew Bassett (<i>Wellcome Sanger Institute</i>) Dr Klio Maratou (<i>GlaxoSmithKline</i>) Dr Marcello Maresca (<i>AstraZeneca</i>)
11:05 - 11:50	PLENARY KEYNOTE Examining intrinsic and extrinsic regulation of human brain development using cerebral organoids Dr Madeline Lancaster (<i>MRC Laboratory of Molecular Biology, Cambridge</i>)
11:50 - 12:20	IOTA SCIENCES PLATINUM SPONSOR Highly efficient single-cell cloning of gene-edited human iPSCs in sub-microliter volumes Dr Katia Mattis
12:20 - 13:20	REFRESHMENT BREAK, POSTER SESSION AND VENDOR EXHIBITION
13:20 - 13:25	SESSION CHAIR INTRODUCTIONS Dr Klio Maratou (<i>GlaxoSmithKline</i>)
13:25 - 13:45	Therapeutic strategies via genome engineering: new approaches and new challenges Prashant Mali (<i>UCSD</i>)
13:45 - 14:05	Deciphering the pathogenicity of cardiac disease-associated genetic variants using human iPSCs and gene editing Dr Richard Davis (<i>Leiden University Medical Center</i>)
14:05 - 14:20	NEXCELOM BIOSCIENCE LTD INDUSTRY INSIDER High-throughput detection of CRISPR/Cas 9 gene editing efficiency, cell proliferation, viability, and monoclonality validation using image cytometry Dr Sun Yung
14:20 - 14:30	REFRESHMENT BREAK
14:30 - 14:50	Cell engineering at the speed of light Dr Greg Sienski (<i>AstraZeneca</i>)
14:50 - 15:10	Joint Q&A session Prashant Mali (<i>UCSD</i>) Dr Richard Davis (<i>Leiden University Medical Center</i>) Dr Greg Sienski (<i>AstraZeneca</i>)
15:10 - 15:25	BIT.BIO INDUSTRY INSIDER CRISPR and the Art of Perturbation Screening: Unbiased functional genomic screening meets the best human cellular models Kam Dhaliwal
15:10 - 15:35	REFRESHMENT BREAK
15:35 - 16:20	PLENARY KEYNOTE Editing bone marrow stem cells to cure blood disorders Prof Jacob Corn (<i>ETH Zürich</i>)
16:20 - 16:40	Mechanistic Insights into Cytosine and Adenine Base Editors Prof Alexis Komor (<i>UCSD</i>)
16:40 - 16:45	REFRESHMENT BREAK
16:45 - 17:05	Expanding gene-editing tool delivery and targeting range in human cells Manuel Goncalves (<i>LMUC</i>)
17:05 - 17:25	Strategies to improve precision and safety of human cells manipulation Dr Claudio Mussolino (<i>University of Freiburg</i>)
17:25 - 17:45	Joint Q&A session Prof Alexis Komor (<i>UCSD</i>) Manuel Goncalves (<i>LMUC</i>) Dr Claudio Mussolino (<i>University of Freiburg</i>)
IMproving GeNome Editing Efficiency (IMGENE) European Training Network presentations	
17:45 - 17:50	Modulation of DNA repair pathways by HDR-CRISPR promotes seamless genome editing in primary human hematopoietic cells Antonio Carusillo (<i>Center for Transfusion Medicine and Gene Therapy</i>)
17:50 - 17:55	Homology-directed safe harbour insertion of transgenes encoding full-length dystrophin through high-capacity adenoviral vector delivery of CRISPR-Cas9 nucleases and donor DNA Francesca Tasca (<i>LMUC</i>)
17:55 - 18:00	What do people think about genetic engineering: a systematic review of post-CRISPR questionnaires Pedro Dias Ramos (<i>IBMC</i>)
18:00 - 18:05	Joint Q&A session Francesca Tasca (<i>LMUC</i>) Antonio Carusillo (<i>Center for Transfusion Medicine and Gene Therapy</i>) Pedro Dias Ramos (<i>IBMC</i>)
18:05 - 18:15	Conference Close & Poster Winner Presentations Dr Marcello Maresca (<i>AstraZeneca</i>)

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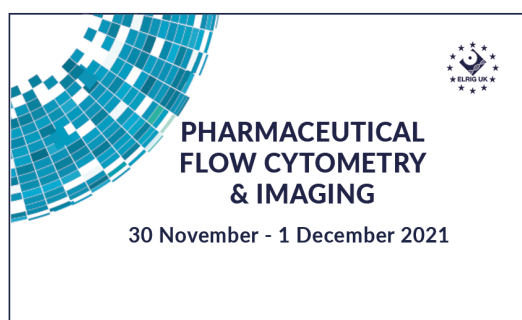
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