## **CRISPRMED25 Final Programme**

- CRISPRMED25 Programme digital version click <u>here</u>. Please note that the digital programme is the most up-to-date version.
- Abstract Section click <u>here</u> (only visible for registrants)
- Participant Section click <u>here</u> (only visible for registrants)

Monday, 07 April 2025

15:00 - 17:15	CRISPR Medicine Conference 2025 Virtual: Oral session – more de	etail <u>s</u>
	Transformative Gene Editing: From Rare Disease Models to Clinical	
	Fetal gene therapy in a mouse model of Krabbe disease using	
	virus-like particles   <u>Asma Naseem</u> <sup>1</sup>	
	UCL Great Ormond Street Institute of Child Health, UK	
	Transforming cardiometabolic disease treatment paradigm:	<u>ID 143</u>
	Advancing in vivo CRISPR-based therapies to target key	
	dyslipidemia drivers   <u>Maria Mirotsou</u> <sup>1</sup>	
	In vivo genome editing: Translating science from bench to bedside	<u>ID 131</u>
	Emma Wang <sup>1</sup> YolTech Therapeutics, China	
	Achieving non-viral high-fidelity gene editing and gene insertion:	<u>ID 140</u>
	updates from Poseida Therapeutics   Blair Madison <sup>1</sup>	
	Clinical progress of off-the-shelf CAR-T cell therapies engineered	ID 125
	with CRISPR hybrid RNA-DNA (chRDNA) genome-editing	
	technology   <u>Tina Albertson</u> <sup>1</sup> 'Caribou Biosciences, Inc., US	
17:15 - 18:00	CRISPR Medicine Conference 2025 Virtual: Poster session – more	details
	Precise Gene Writing System for CAR-T cell therapy generation	<u>ID 7</u>
	Paolo Petazzi¹ Integra Therapeutics, Spain	
	Democratizing CRISPR in its public-private ecosystem	<u>ID 8</u>
	Franziska Bächler <sup>1</sup>  University of Basel	
	Designing and cloning of single guide RNA for green fluorescent	<u>ID 14</u>
	protein	
	Mahla Sadat Hosseini <sup>1, 2</sup>	
	Department of Stem Cells and Developmental Biology, Cell Science Research Centre, Royan Institute for Stem Cell Biology and Technology, ACECR, Tehran, Iran, <sup>2</sup> Department of Basic and Population Based Studies in NCD, Reproductive Epidemiology Research Center, Royan Institute, ACECR, Tehran, Iran	
	Efficient genome editing using 'NanoMEDIC' AsCas12a-VLPs	<u>ID 32</u>
	produced with Pol II-transcribed crRNA	
	Natalia Kruglova <sup>1, 2</sup> Institute of Gene Biology Russian Academy of Sciences, 119334 Moscow, Russia, <sup>2</sup> Center for Precision Genome Editing and Genetic	
	Technologies for Biomedicine, Institute of Gene Biology Russian Academy of Sciences, 119334 Moscow, Russia	ID 04
	Engineering tripartite gene editing machinery for highly efficient	<u>ID 34</u>
	non-viral targeted genome integration	
	Hao Wu¹ Whitehead institute, US	
	A Simplified method to Custom Transgenic Mouse Models Using	<u>ID 44</u>
	CRISPR-RNPs through Virus-Like Particles	
	Da Eun Yoon <sup>1, 2, 3</sup>	
	<sup>1</sup> Transgenic core facility, Max-Planck Institute of Biochemistry, <sup>2</sup> Department of Physiology, Korea University College of Medicine, <sup>3</sup> Department of Biomedical Sciences, Korea University College of Medicine	
	Cas9 mRNA and its aggregate analysis on SEC HPLC	<u>ID 102</u>
	Konstantin Musiychuk <sup>1</sup>  Sepax Technologies, US	
	Genome-wide off-target assessment of adenine base editing:	<u>ID 108</u>
	Establishing a WGS pipeline for safe gene correction	
	Aidin Kazemizadeh <sup>1</sup>  King's College London, UK	

Tuesday, 08 April 2025

<b>Tuesday, 08 A</b> 09:00 - 13:25	CRISPRMED25 Workshops – more <u>details</u>	
09:00 - 13:00	Founders' Breakfast – more <u>details</u>	
11:00 -	CRISPRMED25 In-Person Conference Registration Opens	
14:00 - 14:15	CRISPRMED25 Opening Address by CRISPR Medicine News	
14:15 - 15:00	Patient Voice	
	Scaling the mountain: Expanding gene therapy to underserved communities   Jimi Olaghere	<u>ID 52</u>
15:00 - 16:00	Keynote Address	
	CRISPR-Cas: From bacterial immunity towards genome editing and beyond   Virginijus Šikšnys¹	ID 121
16:00 - 16:30	'Vilnius University Life Sciences Centre, Lithuania  Coffee Break	
16:30 - 19:00	Genome Editing: From Pioneering Foundations to Modern Applications	
	Foundations of genome editing: Past, present and future    Dana Carroll¹  !University of Utah, US	<u>ID 20</u>
	Origins and applications of genome editing    Srinivasan Chandrasegaran  Uohns Hookins School of Public Health, US	ID 9
	A universal CRISPR analytical platform for precision monitoring of CRISPR reagents and editing efficiency   Kiana Aran <sup>1</sup>	<u>ID 133</u>
	RNA base editing with endogenous ADAR   Thorsten Stafforst <sup>1</sup> 'Interfaculty Institute of Biochemistry; University of Tübingen, Germany Gene and RNA Therapy Center, University Hospital, Faculty of Medicine, Tübingen, Germany	<u>ID 11</u>
	From linear synthesis to ligation: Comparative insights into long nucleic acid production (sponsored)   Philipp Kamm <sup>1</sup>	<u>ID 130</u>
19:00 - 21:00	Poster and Networking Session 1	

Wednesday, 09 April 2025

06:30 - 07:15	Fun Run in Copenhagen (sponsored by Vivlion)	
08:00 - 10:05	Clinical Implementation of Gene-Editing Therapies: Progress and Solution	ons
	Refining tumor-infiltrating lymphocytes for adoptive cell therapy in solid tumors   Özcan Met <sup>1, 2</sup> 'National Center for Cancer Immune Therapy (CCIT-DK), Department of Oncology, Copenhagen University Hospital, Herlev, Denmark,	<u>ID 120</u>
	Paving the way to a single source for gene editing with strategic expansions (sponsored)   Amanda Haas	<u>ID 118</u>
	CRISPR-directed gene editing for head and neck cancer, a patient centric approach   Eric Kmiec <sup>1</sup> 'Gene Editing Institute at ChristianaCare, Delaware, US	<u>ID 13</u>
	Next-generation CRISPR genome editing to combat fatal genetic diseases and extend lifespan   Alvin Luk <sup>2, 3</sup> *HuidaGene Therapeutics, *Cholgene Therapeutics, China	<u>ID 49</u>
	Accelerating cell and gene therapy success with cGMP-compliant gene editing components (sponsored)   <u>Juliana Campo</u> <sup>1</sup>	<u>ID 123</u>
	Large scale manufacturing of a hematopoietic stem cell gene editing-based protocol for Pyruvate kinase deficiency therapy (short talk)   Jose-Carlos Segovia <sup>1</sup> 'Cell Technology Division, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER); Unidad Mixta de Terapias Avanzadas. Instituto de Investigación Sanitaria Fundación Jiménez Diaz (IIS-FJD, UAM), Madrid, Spain	<u>ID 27</u>
	PathCrisp and EdiCrisp: CRISPR-powered innovations for enhanced diagnostics and therapeutics (short talk)   <u>BS Kruthika</u> <sup>1, 2</sup> 'Crisprbits Private limited, 'Crisprbits laboratory	<u>ID 54</u>
10:05 - 10:30	Coffee Break	
10:30 - 11:30	Panel Discussion: Patient Access to CRISPR Medicine – More <u>details</u>	
11:30 - 12:00	Beyond Conventional CRISPR-Based Genome Editing	
	CRISPR/Cas transcriptional gene regulation in hematopoietic cells  Rasmus Bak¹  Department of Biomedicine, Aarhus University, Denmark	<u>ID 114</u>
	Decoding the molecular mechanisms of CRISPR-associated transposons to reset genome engineering   Guillermo Montoya <sup>1</sup> 'University of Copenhagen, Denmark	<u>ID 135</u>
	Improving CRISPR base editing efficiency prediction through data generation and deep learning (short talk)   Ying Sun <sup>3</sup> *Center for non-coding RNA in Technology and Health, Department of Veterinary and Animal Sciences, Faculty of Health and Medical Sciences, University of Copenhagen, Frederiksberg, Denmark	<u>ID 76</u>
	Engineering a potent CRISPR-Cas12l genome editor with C-rich PAM recognition (Industry Talk)  Giedrius Gasiunas  Casazyme	<u>ID 75</u>
13:00 - 13:45	Lunch	
13:45 - 15:45	Understanding the Outcomes of Genome Editing	
	Single-cell profiling of genome-editing alterations and functional outcomes in CRISPR-engineered cells   Ayal Hendel <sup>1</sup>	<u>ID 117</u>
	Evolving solutions for effective genotoxicity assessment in  CRISPR-based editing (sponsored)   Ashley Jacobi¹  Integrated DNA Technologies, US	<u>ID 97</u>
	Genome editing with AZD7648 induces large-scale genomic alterations   Gregoire Cullot <sup>3</sup> *Department of Biology, Institute of Molecular Health Sciences, ETH Zurich, Zurich, Switzerland	ID 24

	Precision genotyping tools for gene editing analysis and	<u>ID 142</u>
	characterization (sponsored)   <u>Iván Hernández-Neuta</u> <sup>1</sup>	
	Modelling CRISPR-Cas9 kinetics reveals recurrent cleavage,	<u>ID 81</u>
	precise repair, and chromosomal aberration dynamics (short talk)  Alexander Chalk  PatraZaneca, Sweden	
	INDUCE-seq®: Ensuring the safe development of cell and gene	ID 113
	therapies by gene editing (sponsored)   Simon Reed  Broken String Biosciences, UK	
	Cell-type and time-resolved genotoxicity of base editing (short talk)  Sandra Ammann <sup>2</sup>	ID 90
45:45 40:45	<sup>2</sup> Institute for Transfusion Medicine and Gene Therapy, Medical Center - University of Freiburg, Freiburg, Germany	BA BA
15:45 - 16:45	Panel Discussion: Navigating Regulatory Pathways for CRISPR	Medicine – More <u>details</u>
16:45 - 17:15	Coffee Break	
17:15 - 19:00	Functional Genomics - In Vivo and In Vitro CRISPR Screens	
	Dissecting intracellular signaling with base editor screens  Jakob Nilsson <sup>1</sup>	ID 58
	PDanish Cancer Institute, Denmark  Advancing CRISPR Applications in Precision Oncology  Rob Wolthuis <sup>1</sup>	<u>ID 60</u>
	'Amsterdam UMC, Netherlands	10.00
	Revealing neuronal vulnerabilities with direct in vivo AAV-mediated CRISPR screens   <u>Alessio Strano</u> <sup>1</sup>	ID 39
	The next frontier in functional genomics: design, execution and analysis of combinatorial CRISPR screens (sponsored)	ID 128
	Manuel Kaulich <sup>1</sup> Vivlion GmbH and Goethe University Frankfurt, Germany	
	CRISPR-based functional genomics for AAV production (short talk)  Alessandra Recchia  University of Modena and Reggio Emilia, Italy	<u>ID 50</u>
19:00 - 21:00	Exhibitor Evening	!
	An evening of fun and games with food and beverages!	

Thursday, 10 April 2025

06:30 - 07:15	Fun Run in Copenhagen		
08:00 - 10:00	Advances in Gene-Edited Cell Therapies		
	Novel editing approaches to beta-hemoglobinopathies  Annarita Miccio  Imagine Institute, France	<u>ID 62</u>	
	Genome editing by homology directed repair (HDR) to create stem cell based drugs   Matthew Porteus MD, PhD¹  'Sutardja Chuk Professor of Definitive and Curative Medicine Department of Pediatrics Institute of Stem Cell Biology and Regenerative	<u>ID 116</u>	
	Medicine Stanford University, US  Mechanistic insights to further advance gene and cell therapy applications   Raffaella Di Micco  Applications   Raffaella Di Micco	<u>ID 46</u>	
	Fuelling cancer immunotherapy through gene editing  Karim Benabdellah  Genyo, Spain	<u>ID 141</u>	
	Circularization of single-stranded DNA improves TALEN-mediated gene insertion in long term HSC and in primary T cells (short talk)  Julien Valton <sup>1</sup>	ID 3	
	Assessing the safety and applicability of transient p38 inhibition in genome editing protocols for hematopoietic stem and progenitor cells (short talk)   Roberta Vacca <sup>1, 2</sup> San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, 20132, Milan, Italy, <sup>2</sup> Vita-Salute	<u>ID 71</u>	
10:00 - 10:25	San Raffaele University, 20132, Milan, Italy, Italy  Coffee Break		
10:25 - 12:15	In Vivo Gene Editing For the Treatment of Rare Diseases		
	In vivo gene editing for the treatment of metabolic diseases  Alessia Cavazza <sup>1, 2</sup> Molecular and Cellular Immunology section, Department of Infection, Immunity & Inflammation, UCL Great Ormond Street Institute of Child Health, University College London, 20 Guilford Street, WC1N 1DZ, London, UK, <sup>2</sup> Department of Medical and Surgical Sciences for	<u>ID 122</u>	
	Children and Adults, University of Modena and Reggio Emilia School of Medicine, Via del Pozzo 71, 41125, Modena, Italy  Correcting genetic diseases by in vivo base- and prime editing  Gerald Schwank  Institute of Pharmacology and Toxicology, University of Zurich, Switzerland	<u>ID 138</u>	
	Dual precise repair of disease-causing mutations in compound  heterozygous muscular dystrophy (short talk)   Helena Escobar <sup>2</sup> Muscle Research Unit, Experimental and Clinical Research Center (ECRC), a joint cooperation between the Charité -	<u>ID 105</u>	
	Universitätsmedizin Berlin and the Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC), Berlin, Germany  Lung targeting lipid nanoparticle (LNP) delivery for cystic fibrosis  (short talk)   Marco S. Weinberg <sup>1</sup> 'ReCode Therapeutics, US	<u>ID 95</u>	
	EDSpliCE: AAV-deliverable enhanced deletion RNA-guided nucleases for therapeutic splicing modulation - applied to Usher syndrome (short talk)   Salome Spaag <sup>1</sup>	<u>ID 80</u>	
	'Institute for Ophthalmic Research, University Hospital Tübingen, Germany  Adenine base editors as a breakthrough in advanced genetic  therapy for COL6-RD (short talk)   Arístides López-Márquez   'Institut de Recerca Sant Joan de Deu, Spain	<u>ID 17</u>	
12:15 - 13:30	Special Session: Democratising Genetic Medicine – More <u>details</u>		
	Genome engineering education and capacity building in Africa  Thomas O. Auer <sup>1, 2</sup> 'University of Fribourg, Department of Biology, Switzerland, <sup>2</sup> TReND in Africa, UK Gene4all, Spain	ID 137	
	Equity in health and diverse genomes: Unveiling Africa's potential  Segun Fatumo  Queen Mary University of London, UK	<u>ID 136</u>	
	Panel discussion: Advancing equitable access to genetic technologies in research and healthcare  Vincenzo Di Donato  Gene4AII	<u>ID 144</u>	

13:30 - 14:15	Lunch	
14:15 - 16:00	Innovations in Gene-Editing Delivery Systems	
	Harnessing a diverse collection of CRISPR-associated RNA-guided nucleases and a proprietary nonviral delivery platform for precise gene editing   Lilian Lamech <sup>1</sup>	<u>ID 77</u>
	Development of SORT lipid nanoparticles (LNPs) for genome correction of disease-causing mutations   Daniel Siegwart    University of Texas Southwestern Medical Center, US	<u>ID 48</u>
	Engineered lentivirus-derived particles for in vivo CRISPR RNP delivery and prime editing   <u>Jacob Giehm Mikkelsen</u> ¹	<u>ID 115</u>
	Al-designed protein binders for gene editor modulation and AAV retargeting   Martin Pacesa <sup>1</sup>	<u>ID 6</u>
	Enabling production of next-generation genetic medicines through comprehensive CRISPR manufacturing solutions (sponsored)  Max Sellman  Faldewron, US	<u>ID 127</u>
16:00 - 17:00	Special Session: The Path to the First Genome-Editing Trial – More	<u>details</u>
17:00 - 17:30	Coffee Break	
17:30 - 19:30	CRISPRMED25 Event with WeDoCRISPR Poster Flash Talks	

Friday, 11 April 2025

06:30 - 07:15	Fun Run in Copenhagen		
08:50 - 11:00	Technological Advances Within In Vivo Genome Editing		
	ePsCas9: A robust Cas9 with SpOT-ON precision for therapeutic	<u>ID 132</u>	
	genome editing		
	Marcello Maresca  l'AstraZeneca, Sweden		
	Genome-wide activity of a CRISPR/Cas9 system that targets	<u>ID 18</u>	
	collagen VI mutations		
	Cecilia Jimenez-Mallebrera  Levita de Bourse Serbier de Blu Marital Serbiera de Blu Barralera Cesia		
	Institut de Recerca Sant Joan de Déu. Hospital Sant Joan de Déu Barcelona, Spain  Engineering CRISPR technologies for cardiovascular medicine  Julian Grünewald <sup>1</sup>	<u>ID 66</u>	
	¹TUM, Germany	ID 05	
	Long-circulating lipid nanoparticles (IcLNP) effectively deliver nucleic	<u>ID 85</u>	
	acids to hematopoietic stem and progenitor cells in the bone marrow		
	(short talk)   <u>Jessica Silva</u> ²		
	Optimisation of advanced gene editing techniques and their	ID 36	
	application in the treatment of cardiovascular diseases (short talk)  Sayari Bhunia <sup>1, 2, 3</sup>		
	Institute of Pharmacology, Heidelberg University, Heidelberg, Germany, <sup>2</sup> DZHK (German Center for Cardiovascular Research), Partner Site Heidelberg/ Mannheim, Heidelberg University, Heidelberg, Germany, <sup>3</sup> Heidelberg Biosciences International Graduate School		
	Harnessing bacteriophage vectors to deliver CRISPR tools for	ID 139	
	targeted microbiome therapies   Antoine Decrulle <sup>1</sup>		
	'Eligo Bioscience, France		
11:00 - 11:25	Coffee Break		
11:25 - 13:15	Functional Genomics - Discovery and Function Studies		
	Systematic surveys of synthetic lethality with the CRISPR/Cas12a	<u>ID 12</u>	
	IN4MER platform   <u>Travor Hart PhD</u> ¹		
	'Associate Professor, Dept of Systems Biology University of Texas MD Anderson Cancer Center, US	ID 440	
	The effect of genetic perturbations on bioactivity across modalities  James Longden  FEVOIVUS	<u>ID 119</u>	
	A proteome-wide dependency map of protein interaction motifs	<u>ID 57</u>	
	(short talk)   Sara M. Ambjørn¹		
	Novo Nordisk Foundation Center for Protein Research, University of Copenhagen, Denmark	ID 424	
	Programmable genome disruption enables selective elimination of cancer cells using a novel CRISPR-Cas nuclease (short talk)	<u>ID 134</u>	
	Michael Krohn <sup>1</sup>		
	<sup>1</sup> Akribion Therapeutics GmbH, Germany	15.71	
	Sensitive dissection of a genomic regulatory landscape using bulk	<u>ID 74</u>	
	and targeted single-cell activation (short talk)   Dubravka Vučićević 1  'Computational Regulatory Genomics, Berlin Institute for Medical Systems Biology of the Max Delbrück Center for Molecular Medicine in the Helmholtz Association, Germany		
	CRISPR/Cas-based approaches using iPSC-derived	<u>ID 21</u>	
	cardiomyocytes and heart spheroids for modelling of Duchenne		
	muscular dystrophy (short talk)   <u>Józef Dulak</u> <sup>1,2</sup>		
	<sup>1</sup> Jagiellonian University, <sup>2</sup> Department of Medical Biotechnology, Faculty of Biochemistry, Biophysics and Biotechnoloy, Jagiellonian University, Kraków, Poland		
13:15 - 13:45	Closing Address by CRISPR Medicine News		
13:45 - 14:00	Light Refreshments		
	OBS: The Cph Canal Trip will be 16:00 – 17:00 for delegates signed up for	r this social	
	activity		