CRISPRMED25 Final Programme

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- 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	details	
	Transformative Gene Editing: From Rare Disease Models to Clinical Application		
	Fetal gene therapy in a mouse model of Krabbe disease using	<u>ID 111</u>	
	virus-like particles <u>Asma Naseem</u> ¹ 'UCL Great Ormond Street Institute of Child Health, UK		
	Transforming cardiometabolic disease treatment paradigm: Advancing in vivo CRISPR-based therapies to target key	<u>ID 143</u>	
	dyslipidemia drivers <u>Maria Mirotsou</u> ¹		
	In vivo genome editing: Translating science from bench to bedside Emma Wang¹ ¹YolTech Therapeutics, China	<u>ID 131</u>	
	Achieving non-viral high-fidelity gene editing and gene insertion:	<u>ID 140</u>	
	updates from Poseida Therapeutics <u>Blair Madison</u> ¹ 'Poseida Therapeutics, US		
	Clinical progress of off-the-shelf CAR-T cell therapies engineered with CRISPR hybrid RNA-DNA (chRDNA) genome-editing	<u>ID 125</u>	
	technology <u>Tina Albertson</u> ¹ 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 Paolo Petazzi Integra Therapeutics, Spain	<u>ID 7</u>	
	Designing and cloning of single guide RNA for green fluorescent	<u>ID 14</u>	
	protein Mahla Sadat Hosseini ^{1, 2} 'Department of Stem Cells and Developmental Biology, Cell Science Research Centre, Royan Institute for Stem Cell Biology and Technology, ACECR, Tehran, Iran, 'Department of Basic and Population Based Studies in NCD, Reproductive Epidemiology Research Center, Royan Institute, ACECR, Tehran, Iran		
	Engineering tripartite gene editing machinery for highly efficient	<u>ID 34</u>	
	non-viral targeted genome integration <u>Hao Wu</u> ¹		
	Cas9 mRNA and its aggregate analysis on SEC HPLC Konstantin Musiychuk Sepax Technologies, US	<u>ID 102</u>	
	Genome-wide off-target assessment of adenine base editing: Establishing a WGS pipeline for safe gene correction Aidin Kazemizadeh Kings College London, UK	<u>ID 108</u>	

Tuesday, 08 April 2025

Tuesday, 08 A	pril 2025	
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	<u>ID 52</u>
	communities <u>Jimi Olaghere</u>	
15:00 - 16:00	Keynote Address	
	CRISPR-Cas: From bacterial immunity towards genome editing and	<u>ID 121</u>
	beyond <u>Virginijus Šikšnys</u> ¹	
10.00 10.00	Vilnius University Life Sciences Centre, Lithuania	
16:00 - 16:30	Coffee Break	
16:30 - 19:00	Genome Editing: From Pioneering Foundations to Modern Applications	
	Foundations of genome editing: Past, present and future	ID 20
	Dana Carroll ¹ 'University of Utah, US	
	Origins and applications of genome editing	<u>ID 9</u>
	Srinivasan Chandrasegaran ¹ 'Johns Hopkins School of Public Health, US	
	A universal CRISPR analytical platform for precision monitoring of	<u>ID 133</u>
	CRISPR reagents and editing efficiency Kiana Aran ¹	
	RNA base editing with endogenous ADAR Thorsten Stafforst ¹	<u>ID 11</u>
	^¹ Interfaculty Institute of Biochemistry; University of Tübingen, Germany Gene and RNA Therapy Center, University Hospital, Faculty of Medicine, Tübingen, Germany	
	From linear synthesis to ligation: Comparative insights into long	<u>ID 130</u>
	nucleic acid production (sponsored) Philipp Kamm ¹	
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 ^{1, 2} '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	ID 118
	CRISPR-directed gene editing for head and neck cancer, a patient centric approach Eric Kmiec ¹ '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 ^{2, 3} *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> ¹	<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 ¹ '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> ^{1, 2} '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 ¹ 'University of Copenhagen, Denmark	<u>ID 135</u>
	Improving CRISPR base editing efficiency prediction through data generation and deep learning (short talk) Ying Sun ³ *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 ¹	<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 ³ *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> ¹ Countagen AB, Sweden	
	Modelling CRISPR-Cas9 kinetics reveals recurrent cleavage, precise repair, and chromosomal aberration dynamics (short talk) Alexander Chalk [AstraZaneca, Sweden]	ID 8
	INDUCE-seq®: Ensuring the safe development of cell and gene therapies by gene editing (sponsored) Simon Reed¹	ID 11:
	Cell-type and time-resolved genotoxicity of base editing (short talk) Sandra Ammann ² Institute for Transfusion Medicine and Gene Therapy, Medical Center - University of Freiburg, Freiburg, Germany	ID 90
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 ¹ Danish Cancer Institute. Denmark	ID 58
	Targeting tumor specific copy number alterations using CRISPR-Kill Rob Wolthuis Amsterdam UMC, Netherlands	ID 6
	Revealing neuronal vulnerabilities with direct in vivo AAV-mediated CRISPR screens Alessio Strano ¹ Department of Biosystems Science and Engineering, ETH Zurich, 4056 Basel, Switzerland	ID 3
	The next frontier in functional genomics: design, execution and analysis of combinatorial CRISPR screens (sponsored) Manuel Kaulich Vivilion GmbH and Goethe University Frankfurt, Germany	ID 128
	CRISPR-based functional genomics for AAV production (short talk) Alessandra Recchia University of Modena and Reggio Emilia, Italy	<u>ID 5</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	ID 62
	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 1	<u>ID 46</u>
	Fuelling cancer immunotherapy through gene editing Karim Benabdellah Genvo, 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 Cellectis SA, France	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 ^{1, 2} 'San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, 20132, Milan, Italy, *Vita-Salute	ID 71
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 ^{1, 2} '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, '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 ¹	<u>ID 138</u>
	'Institute of Pharmacology and Toxicology, University of Zurich, Switzerland Dual precise repair of disease-causing mutations in compound heterozygous muscular dystrophy (short talk) Helena Escobar ² 'Muscle Research Unit, Experimental and Clinical Research Center (ECRC), a joint cooperation between the Charité -	ID 105
	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 ¹ (ReCode Therapeutics, US	ID 95
	EDSpliCE: AAV-deliverable enhanced deletion RNA-guided nucleases for therapeutic splicing modulation - applied to Usher syndrome (short talk) Salome Spaag ¹	<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 ^{1, 2} 'University of Fribourg, Department of Biology, Switzerland, ² TReND in Africa, UK Gene4all, Spain	<u>ID 137</u>
	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 GenedAll	<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 ¹	<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 ¹	<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 ¹ PastraZeneca, Sweden		
	Genome-wide activity of a CRISPR/Cas9 system that targets	<u>ID 18</u>	
	collagen VI mutations		
	Cecilia Jimenez-Mallebrera ¹ Institut de Recerca Sant Joan de Déu. Hospital Sant Joan de Déu Barcelona, Spain		
	Engineering CRISPR technologies for cardiovascular medicine Julian Grünewald ¹	<u>ID 66</u>	
	'TUM, Germany	ID of	
	Long-circulating lipid nanoparticles (lcLNP) 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 FNanoVation Therapeutics		
	Optimisation of advanced gene editing techniques and their	ID 36	
	application in the treatment of cardiovascular diseases (short talk) Sayari Bhunia ^{1, 2, 3}		
	*Institute of Pharmacology, Heidelberg University, Heidelberg, Germany, *DZHK (German Center for Cardiovascular Research), Partner Site Heidelberg/ Mannheim, Heidelberg University, Heidelberg, Germany, *Heidelberg Biosciences International Graduate School		
	Harnessing bacteriophage vectors to deliver CRISPR tools for	ID 139	
	targeted microbiome therapies Antoine Decrulle ¹		
	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 ¹		
	l'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> ^{1, 2}		
	¹ Jagiellonian University, ² 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		