CRISPRMED25 Final Programme

- CRISPRMED25 Programme digital version click <u>here</u>
- 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	<u>etails</u>	
	Transformative Gene Editing: From Rare Disease Models to Clinical Applications		
	Fetal gene therapy in a mouse model of Krabbe disease using virus-	<u>ID 111</u>	
	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 Scribe Therapeutics, US		
	In vivo genome editing: Translating science from bench to bedside Emma Wang¹ 'YolTech Therapeutics, China	<u>ID 131</u>	
	Achieving non-viral high-fidelity gene editing and gene insertion:	ID 140	
	updates from Poseida Therapeutics Blair Madison ¹		
	Clinical progress of off-the-shelf CAR-T cell therapies engineered with CRISPR hybrid RNA-DNA (chRDNA) genome-editing	<u>ID 125</u>	
	technology Tina Albertson 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 non-	<u>ID 34</u>	
	viral targeted genome integration <u>Hao Wu</u> ¹ Whitehead institute, US		
	Cas9 mRNA and its aggregate analysis on SEC HPLC Konstantin Musiychuk¹	<u>ID 102</u>	
	Sepax Technologies, US	ID 100	
	Genome-wide off-target assessment of adenine base editing:	<u>ID 108</u>	
	Establishing a WGS pipeline for safe gene correction Aidin Kazemizadeh 1		
	'King's College London, UK		

Tuesday, 08 April 2025

Tuesday, 08 A	•	
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	ID 52
	communities <u>Jimi Olaghere</u>	
15:00 - 16:00	Keynote Address	
	CRISPR-Cas: From bacterial immunity towards genome editing and	ID 121
	beyond <u>Virginijus Šikšnys</u> 1 'Vilnius University Life Sciences Centre, Lithuania	
16:00 - 16:30	Coffee Break	
16:30 - 18:30	Genome Editing: From Pioneering Foundations to Modern Application	ons
	Foundations of genome editing: Past, present and future Dana Carroll¹ University of Utah, US	<u>ID 20</u>
	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 'University of California San Diego, US	
	From linear synthesis to ligation: Comparative insights into long	<u>ID 130</u>
	nucleic acid production (sponsored) Philipp Kamm ¹	
18:30 - 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	<u>ID 120</u>	
	Solid tumors Özcan Met ^{1, 2} National Center for Cancer Immune Therapy (CCIT-DK), Department of Oncology, Copenhagen University Hospital, Herley, Denmark,		
	Department of Health Technology, Technical University of Denmark, Lyngby, Denmark	ID 440	
	Paving the way to a single source for gene editing with strategic	<u>ID 118</u>	
	expansions (sponsored) <u>Amanda Haas</u> ¹ 'Agilent Technologies, US		
	CRISPR-directed gene editing for head and neck cancer, a patient	<u>ID 13</u>	
	centric approach Eric Kmiec¹		
	Gene Editing Institute at ChristianaCare, Delaware, US Next-generation CRISPR genome editing to combat fatal genetic	ID 49	
	diseases and extend lifespan Alvin Luk ^{2, 3}		
	² HuidaGene Therapeutics, ³ Cholgene Therapeutics, China		
	Accelerating cell and gene therapy success with cGMP-compliant	<u>ID 123</u>	
	gene editing components (sponsored) Juliana Campo ¹ GenScript Biotech, Netherlands		
	Large scale manufacturing of a hematopoietic stem cell gene	ID 27	
	editing-based protocol for Pyruvate kinase deficiency therapy (short		
	talk) <u>Jose-Carlos Segovia</u> 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 Díaz (IIS-FJD, UAM), Madrid, Spain PathCrisp and EdiCrisp: CRISPR-powered innovations for	ID 54	
	enhanced diagnostics and therapeutics (short talk) BS Kruthika ^{1, 2}	<u> </u>	
	Crisprbits Private limited, ² Crisprbits laboratory		
10:05 - 10:30	Coffee Break		
10:30 - 12:00	Beyond Conventional CRISPR-Based Genome Editing		
	CRISPR/Cas transcriptional gene regulation in hematopoietic cells	ID 114	
	Rasmus Bak ¹		
	Department of Biomedicine, Aarhus University, Denmark	ID 11	
	RNA base editing with endogenous ADAR Thorsten Stafforst ¹ University of Tübingen, Germany Gene and RNA Therapy Center, University Hospital, Faculty of	<u>15 11</u>	
	Medicine, Tübingen, Germany	ID 405	
	Decoding the molecular mechanisms of CRISPR-associated	<u>ID 135</u>	
	transposons to reset genome engineering Guillermo Montoya ¹ University of Copenhagen, Denmark		
	Improving CRISPR base editing efficiency prediction through data	<u>ID 76</u>	
	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		
	Efficient genome editing using 'NanoMEDIC' AsCas12a-VLPs	ID 32	
	produced with Pol II-transcribed crRNA (short talk)		
	Natalia Kruglova Center for Precision Genome Editing and Genetic Technologies for Biomedicine, Institute of Gene Biology Russian Academy of		
12:00 - 13:00	Panel Discussion: Patient Access to CRISPR Medicine – More details		
13:00 - 13:45	Lunch		
13:45 - 15:45	Understanding the Outcomes of Genome Editing		
	Single-cell profiling of genome-editing alterations and functional	<u>ID 117</u>	
	outcomes in CRISPR-engineered cells Ayal Hendel		
	Bar Ilan University, Israel	ID 07	
	Evolving solutions for effective genotoxicity assessment in CRISPR-	<u>ID 97</u>	
Î	based editing (sponsored) Ashley Jacobi ¹		

	Genome editing with AZD7648 induces large-scale genomic	<u>ID 24</u>	
	alterations Gregoire Cullot ³		
	³ Department of Biology, Institute of Molecular Health Sciences, ETH Zurich, Zurich, Switzerland		
	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,	ID 8 ²	
		<u>ID 6</u>	
	precise repair, and chromosomal aberration dynamics (short talk) Alexander Chalk ¹		
	'AstraZaneca, Sweden		
	INDUCE-seq®: Ensuring the safe development of cell and gene	<u>ID 11:</u>	
	therapies by gene editing (sponsored) <u>Simon Reed</u> ¹ 'Broken String Biosciences, UK		
	Cell-type and time-resolved genotoxicity of base editing (short talk)	<u>ID 90</u>	
	Sandra Ammann ²		
15:45 - 16:45	Institute for Transfusion Medicine and Gene Therapy, Medical Center - University of Freiburg, Freiburg, Germany Panel Discussion: Navigating Regulatory Pathways for CRISPR Medicine	- More details	
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	<u>ID 58</u>	
	Jakob Nilsson ¹ Danish Cancer Institute, Denmark		
	Targeting tumor specific copy number alterations using CRISPR-Kill Rob Wolthuis FAMSterdam UMC. Netherlands	<u>ID 60</u>	
	Revealing neuronal vulnerabilities with direct in vivo AAV-mediated	ID 3	
	CRISPR screens Alessio Strano¹		
	Department of Biosystems Science and Engineering, ETH Zurich, 4056 Basel, Switzerland		
	The next frontier in functional genomics: design, execution and	<u>ID 12</u>	
	analysis of combinatorial CRISPR screens (sponsored)		
	Manuel Kaulich ¹ Vivlion GmbH and Goethe University Frankfurt, Germany		
	CRISPR-based functional genomics for AAV production (short talk)	ID 50	
	Alessandra Recchia ¹		
19:00 - 21:00	University of Modena and Reggio Emilia, Italy Exhibitor Evening		
19:00 - 21:00	University of Modena and Reggio Emilia, Italy Exhibitor Evening		

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 Annarita Miccio	<u>ID 62</u>
	Genome editing by homology directed repair (HDR) to create stem	<u>ID 116</u>
	Cell based drugs Matthew Porteus MD, PhD Sutardja Chuk Professor of Definitive and Curative Medicine Department of Pediatrics Institute of Stem Cell Biology and Regenerative Medicine Stanford University, US	
	Mechanistic insights to further advance gene and cell therapy applications Raffaella Di Micco	<u>ID 46</u>
	Fuelling cancer immunotherapy through gene editing Karim Benabdellah ¹	<u>ID 141</u>
	Circularization of single-stranded DNA improves TALEN-mediated	ID 3
	gene insertion in long term HSC and in primary T cells (short talk) Julien Valton Cellectis SA, France	
	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}	<u>ID 71</u>
	San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, 20132, Milan, Italy, ² Vita-Salute San Raffaele University, 20132, Milan, Italy, Italy	
10:00 - 10:25	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, ² 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 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 ² *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 ¹	<u>ID 95</u>
	EDSpliCE: AAV-deliverable enhanced deletion RNA-guided nucleases for therapeutic splicing modulation - applied to Usher syndrome (short talk) Salome Spaag ¹	ID 80
	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 details	
	Genome engineering education and capacity building in Africa Thomas O. Auer 1, 2 University of Fribourg, Department of Biology, Switzerland, 2TReND 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	ID 136
	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>
	Life Edit Therapeutics, an ElevateBio Company, US 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 Addevon, US	<u>ID 127</u>
16:00 - 17:00	Special Session: The Path to the First Genome-Editing Trial – More	e <u>details</u>
17:00 - 17:30	Coffee Break	
17:30 - 19:00	WeDoCRISPR Poster Flash Talks	
19:00 - 22:00	CRISPRMED25 Evening	

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	
	AstraZeneca, Sweden Genome-wide activity of a CRISPR/Cas9 system that targets	ID 18
	collagen VI mutations	<u></u>
	Cecilia Jimenez-Mallebrera ¹	
	Institut de Recerca Sant Joan de Déu. Hospital Sant Joan de Déu Barcelona, Spain	
	Engineering CRISPR technologies for cardiovascular medicine	ID 66
	Julian Grünewald TUM, Germany	
	Long-circulating lipid nanoparticles (lcLNP) effectively deliver nucleic	ID 85
	acids to hematopoietic stem and progenitor cells in the bone marrow	
	(short talk) <u>Jessica Silva</u> ²	
	NanoVation 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, ² DZHK (German Center for Cardiovascular Research), Partner Site Heidelberg/ Mannheim, Heidelberg University, Heidelberg, Germany, ³ Heidelberg Biosciences International Graduate School	
	(HBIGS), Heidelberg, Germany 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	ID 12
	IN4MER platform <u>Travor Hart PhD</u> ¹	
	Associate Professor, Dept of Systems Biology University of Texas MD Anderson Cancer Center, US	
	The effect of genetic perturbations on bioactivity across modalities	<u>ID 119</u>
	James Longden ¹	
	A proteome-wide dependency map of protein interaction motifs	ID 57
		<u>10 01</u>
	(short talk) Sara M. Ambjørn¹ Novo Nordisk Foundation Center for Protein Research, University of Copenhagen, Denmark	
	Programmable genome disruption enables selective elimination of	ID 134
	cancer cells using a novel CRISPR-Cas nuclease (short talk)	
	Michael Krohn ¹	
	Akribion Therapeutics GmbH, Germany Sensitive dissection of a genomic regulatory landscape using bulk	ID 74
		<u>10 7 -</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
		ulio outlai
	activity	