View Online CRISPRMED24 Program – Click <u>here</u> Access CRISPRMED24 Live App – Click <u>here</u>

### Monday, 22 April 2024 (times in CEST)

15:00 - 16:30	Virtual Event - Selected Presentations Virtual Selected Presentations	
	15:00 - 15:20 Epigenome Editing for the Effective Treatment of HBV	ID 175 Brian Cosgrove Tune Therapeutics
	15:20 - 15:40 Advances and Challenges of Next-Generation CRISPR Gene-Editing Technology for Drug Development	<u>ID 176</u> Alvin Luk HuidaGene Therape
	15:40 - 15:45 GeneAbacusTM: A Novel PCR-free Assay for Gene Editing Validation	<u>ID 186</u> Iván Hernández Countagen
	15:45 - 16:05 A New Approach to Tackle Cancer Using Programmable Cytotoxic Nucleases	ID 182 Michael Krohn Akribion Genomics
	16:05 - 16:25 EMA's Experience & Support to the Development of Medicinal Products Using Genome Editing	<u>ID 181</u> Veronika Jekerle European Medicine
16:30 - 16:40	Virtual Coffee Break	
16:40 - 18:00	Virtual Event - Poster Session Virtual Poster Session	
	Polyplex-based CRISPR/Cas9 In Vivo Mutagenesis for Efficient Lung Cancer Modeling in Rodents	<u>ID 15</u> Mirentxu Santos Biomedical Innova
	CRISPR-Mediated Inactivation of Faah and Magl in Peripheral Nociceptive Neurons: Towards Modulating Endocannabinoid Levels for Pain Control.	<u>ID 17</u> Diana de Prado- Verdún Unidad de Innovac
	Disruption of Epidermal Growth Factor Binding Site by Sequential Knock in CRISPR/Cas9 Genome Editing of Epidermal Growth Factor Receptor and Evaluation of Introduced Mutations in Cervical Cancer Cells	ID 29 Jelena Popovic Radiation Oncolog
	Predicting Off-Target Activity in the CRISPR/Cpf1 System Using Multilayer Perceptron	ID 53 Pragya Kesarwani Regional Centre f
	Enhancing CRISPR-Cas9-Mediated Homology-Directed Repair Using Modified sgRNAs	<u>ID 72</u> Eyemen Kheir University of Trento
	Prenatal In Vivo Base Editing for the Treatment of Krabbe Disease	ID 78 Asma Naseem 1Department of In
	EDSpliCE: A Novel Gene Editing Platform Holding Therapeutic Potential for Splicing Modulation in Inherited Retinal Disorders	<u>ID 83</u> Pietro De Angeli Institute for Oph
	CRISPR/Cas9 Based Knock-In/Knock-Out of RAG2 Gene in Jurkat Cells as Model for Genome Editing Screening	<u>ID 88</u> Zeinab Yousefi- Najafabadi Department of Med

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12:30 - 14:00	Doors will open	
14:00 - 14:15	Welcome and opening address by CMN Main Stage	
14:15 - 15:00	Keynote Lecture - Precision Genetic Engineering of Hematopoiesis k Tools Main Stage	by Gene Editing
	Precision Genetic Engineering of Hematopoiesis by Gene Editing Tools	<u>ID 137</u> Luigi Naldini San Raffaele Tele
15:00 - 15:15	Coffee Break Main Stage	
15:15 - 17:45	<b>Selected talks - one featured presentation from each track</b> Main Stage Chairs: Alessia Cavazza (University College London) and Marcello Maresca (AstraZeneca)	
	15:15 - 15:40 Transforming Gene Therapy From the Few to the Many	<u>ID 152</u> Karina Thorn Novo Nordisk
	15:40 - 16:05 Identification and Evolution of Novel CRISPR-Cas9 Systems From the Human Microbiome	ID 125 Anna Cereseto Department of Cel
	16:05 - 16:30 Connecting Innovators: CA21113 2nd Year Journey in Fostering Collaborative Networks for Breakthroughs in Genome Editing to Treat Human Diseases (GenE-Humdi COST action)	<u>ID 163</u> Karim Benabdellah El Khlanji GENYO, Centre for
	16:30 - 16:55 Precisely Edited Primary Human Muscle Stem Cells as an ATMP in Muscular Dystrophies	ID 145 Simone Spuler Charité Universit
	16:55 - 17:20 New Insights Into On- And Off-Target Effects of Genome Editing Tools	<u>ID 146</u> Toni Cathomen University Freiburg
	17:20 - 17:45 CRISPR-Based Functional Genomic Characterization of Mechanisms of Action of Degraders for Targeted Protein Degradation	<u>ID 142</u> Seung Wook Yang AMGEN
17:45 - 20:00	Poster Session / Networking Session with Snack Poster Area	
17:50 - 18:30	Poster Session A	
	Highly efficient gene editing in human primary T cells using novel CRISPR effectors from the human microbiome	<u>ID 91</u> Antonio Carusillo Alia Therapeutics
	Implementation of TRuC T cells for the treatment of prostate cancer	<u>ID 94</u> Carla Fuster-Garcia

Engineered baculoviral protein and DNA delivery platform for large DNA cargo integration and enhanced gene editing in human cells	ID 95 Francesco Aulicino University of Bri
On- and off-target effects of paired CRISPR-Cas nickase in primary human cells	<u>ID 96</u> Julia Klermund Institute for Tra
Highly efficient and precise gene expression regulation using miniaturized CRISPR-Cas systems	<u>ID 97</u> Yeounsun Oh Department of Lif
Deciphering layers of innate immune regulation by genome-wide CRISPR/Cas9 screens	<u>ID 98</u> Emil Aagaard Thomsen Department of Bio
Harnessing the diversity of CRISPR-Cas proteins for genome editing	<u>ID 100</u> Giedrius Gasiunas CasZyme, Vilnius,
Insights to Cas9-induced host immune activation	<u>ID 101</u> Virpi Stigzelius Clinical Pharmaco
Enzymatic synthesis of ultra-pure long single-stranded DNA to enable Cell and Gene Therapies at scale	<u>ID 102</u> Cosimo Ducani Moligo Technologies
Nomination of Genome-Wide CRISPR-Cas9 Cleavage Activity using rhAmp Technology	<u>ID 103</u> Garrett Rettig Integrated DNA Te
CRISPR-Cas9-Mediated WBP2 Knockout Modulates Tamoxifen Resistance Estrogen Receptor Positive Breast Cancer Cells via the Hippo Pathway	<u>ID 104</u> Ebrucan Bulut Bursa Uludag Univ
EDSpliCE effectively corrects mis-splicing caused by an exonic variant in ABCA4	<u>ID 105</u> Arturo Flores-Tufiño Institute for Oph
GeneAbacus: A novel PCR-free assay for validation of CRISPR-Cas gene editing efficiency with single nucleotide precision	<u>ID 106</u> Felix Neumann Countagen AB, Nob
Engineered VLPs facilitates effective delivery of adenine base editor to correct SNPs in neurons.	Louise Bomholtz Max Delbrück Cent
Gene therapy for neurological diseases - In vitro model of a gene therapy for SynGAP1 syndrome using base editing.	<u>ID 110</u> Daniel Bauersachs Max Delbrück Cent
Enabling CRISPR-Cas associated research through guide RNA manufacturing solutions	ID 111 Ashley Jacobi Integrated DNA Te
Efficient detection of CRISPR/Cas9 bulged off-targets with CRISPRoff2	<u>ID 112</u> Wenhao Gao Center for non-co
Optimized shRNAmir screens unveil cancer vulnerabilities in the essential gene space.	ID 113 Jakub Zmajkovic IMP-Research Inst
High Throughput Single Cell Analysis Workflow for Accurate Measurement of Genotoxicity Arising From Gene Editing Experiments	<u>ID 114</u> Ozcan Sahin Mission Bio
How the Danish Medicines Agency facilitates the entry into the regulatory landscape for advanced therapy medicinal products	Lotte Dahl Nissen Danish Medicines
Efficient sortase-mediated assembly of CRISPR-Cas9	<u>ID 116</u> Seyed Hossein Helalat Technical Univers

Programmable Multi-Kilobase RNA Editing Using CRISPR-Mediated Trans-Splicing	ID 118 Basem Al-Shayeb Amber Bio
Necessity Is the Mother of Innovation, the Ace Pico Protocol Redefining Cell Therapy Delivery	<u>ID 121</u> Dr. Alaa Abdelkarim Mohammed EW ACE Cells Lab UK
New Approach for Designing Split Genome Editors Without Compromising Editing Efficiency	<u>ID 122</u> Jure Bohinc Department of Syn
In Vivo Correction of a Genetically Humanized Fanconi Anemia Mouse Model Using Digital Editing Technologies	<u>ID 123</u> Colette B. Rogers Department of Ped
Less is More: Efficient Novel Non-Viral Immune Cell Engineering With Precise Genomic Integration	<u>ID 134</u> Howard Wu Full Circles Ther
Xdrop®: changing the approach to gene editing validation and single-cell functional assays	<u>ID 143</u> Sidsel Alsing Samplix
INDUCE-seq: Ensuring the safe development of cell and gene therapies by gene editing	<u>ID 144</u> Simon Reed Broken String Bio
Enabling GMP Production of sgRNA for CRISPR-based Cell and Gene Therapies	<u>ID 155</u> Kevin Holden Synthego
Charting New Horizons in guide RNA Manufacturing	<u>ID 156</u> Raoul Hennig BioSpring
Our Karyotyping and FISH Assays as Tools to Evaluate the Genetic Stability of Cell Therapy Products - A Decade of GMP Experience	<u>ID 174</u> Vasileios Georgakakos DR
CRISPR cell based functional assays for supporting pre-clinical drug target development and improved patient stratification	<u>ID 177</u> Arne Nedergaard Kousholt VUS Diagnostics
Software Tools to Enable CRISPR Therapeutics Discovery & Design	<u>ID 179</u> Pawan Patel Benchling
CRISPR Excellence Unleashed: Custom Nuclease Production Services to Propel Your GCT Research Forward!	<u>ID 180</u> Zhengzhi Li Biologics Departm
CRISPR/saCas9 and CRISPR/spCas9 systems for combiatorial genetic screens (CRISPR-KO, CRISPRa, CRISPRi)	<u>ID 183</u> Nadya Isachenko Cellecta, Inc.
CRISPR-Cas9 genome editing system for functional genetic screening in iPSCs	<u>ID 184</u> Nadya Isachenko Cellecta, Inc.
OligoSeq: NextGen Im-/Purity Profiling - Oligo Characterization by Next Generation Sequencing	<u>ID 188</u> Barbara Karolina Pfaff <sub>BioSpring</sub>
Patient-derived Organoids to identify novel combination strategies using CRISPR-based Functional Genomics	ID 189 Dario De Felice AstraZeneca
High Efficiency CRISPR/Cas9 Cell Engineering with MaxCyte Electroporation	<u>ID 191</u> Andrew Mancini MaxCyte
Non-viral DNA Payloads for Gene and Cell Therapy - Total Solutions by GenScript	<u>ID 192</u> Fan Zhou GenScript

Samantha Maragh NIST

#### Overview of the NIST Genome Editing Program

#### 18:30 - 19:15 **Poster Session B**

Development and validation of a novel PD-1/PD-L1 and PD-L2 co- nhibition assay for CRISPR-screening in T cells	ID 4 Beatrice Scapol T-cells and cance
Novel characterization assays enable efficient GMP manufacturing of CRISPR RNP complexes	<u>ID 4</u> Thomas Lync Aldevro
Gene-edited primary muscle stem cells rescue dysferlin-deficient muscular dystrophy	<u>ID 5</u> Helena Escob Charité - Univers
Revolutionizing CRISPR Therapies: A Scalable Manufacturing Platform for eVLPs production	<u>ID 5</u> Lúcia Santo iBET, Instituto d
Predicting Off-Target Activity in the CRISPR/Cpf1 System Using Multilayer Perceptron	<u>ID 5</u> Pragya Kesarwa Regional Centre f
CRISPRBITS- Empowering Health Precision	<u>ID 5</u> Reety Aro CRISPRBIT
Development of a lipidoid library for Cas9-RNP-delivery	<u>ID 5</u> Zian Xu University of Vienr
Initiation of a phase I/II trial of CRISPR-Cas9-mediated PD-1-deficient tumour-infiltrating lymphocyte-based adoptive T-cell therapy in metastatic melanoma	<u>ID 5</u> Michael Crowth National Center f
Base editing of hematopoietic stem cells restores immune function in a mouse model of familial hemophagocytic lymphohistiocytosis	<u>ID 5</u> Lei L Institute for Tra
Development of a CRISPR-based epigenome editing platform to enhance the long-term engraftment of ex-vivo genetically modified HSPCs	<u>ID 5</u> Federica Zinghirir University Colleg
mproved CRISPR/Cas9 Off-target Assessment based on Guide RNA Binding Energy	<u>ID t</u> Dhvani Vo Center for non-Co
Genome-wide CRISPR-Cas9 knockout screen identifies DNA damage response pathways and BTK as essential for cisplatin response in diffuse arge B-cell lymphoma	ID 6 Issa Ismail Iss Department of Hem
Enhanced CRISPR base editing design from data generation and deep earning	<u>ID 6</u> Ying Su Center for non-co
Genome-wide CRISPR screening in postmitotic hepatocytes of murine iver	<u>ID (</u> Joonsun Le Institute of Mole
MyoPax: the vanguard in cell and gene therapies for muscle disorders	<u>ID (</u> Eric Metzler-Bräuch MyoPax Gml
Efficient single-cell cloning and culture of hiPSCs in small-scale fluid- walled cell culture chambers	ID ( Narasimha Telus MaxDelbruck cente
CRISPRon/off: Combined CRISPR/Cas9 on- and off-target design	<u>ID (</u> Christian Antho University of Cop

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9:15 - 20:00	Poster Session C	
	Prime editing-mediated correction of the FANCA gene in primary patient cells	<u>ID 8</u> Karolina Skvarov Kramarzov Childhood Leukaem.
	Exploring the functional impact of IncRNAs through CRISPRi screens in CRC models with KRAS and BRAF mutational background	ID 8 Ante Mihaljevi Department for Bi.
	Gene editing in hematopoietic stem cells by delivery of CRISPR-based genome editing tool kits in lentivirus-derived nanoparticles (LVNPs)	<u>ID 8</u> Sofie Anderse Department of Bio.
	EDSpliCE: A Novel Gene Editing Platform Holding Therapeutic Potential for Splicing Modulation in Inherited Retinal Disorders	ID 8 Pietro De Ange Institute for Oph.
	Prime editing for correction of GATA2 deficiency in human CD34+ hematopoietic stem cells	<u>ID 8</u> Jonas H. Wol Department of Bio.
	CRISPR/Cas9-mediated gene editing delivered by a single AAV vector inhibits viral reactivation of HSV-1 in a latent rabbit keratitis model	<u>ID 8</u> Kevin Lu Excision BioThera.
	Unraveling genome integrity and safety of gene engineered hematopoietic stem and progenitor cells for the treatment of RAG1 deficiency	<u>ID 7</u> Chiara Branda San Raffaele-Tele.
	ePsCas9- a CRISPR tool for in vivo therapeutic genome editing at AstraZeneca	<u>ID 7</u> Aikaterir Emmanouilio Genome Engineerin.
	Generation of an experimental model for whole genome CRISPR screening to study hypoxia-induced drivers of chemotherapy resistance in muscle invasive bladder cancer	Danielle Smit Univeristy of Man.
	Off-targets for good: Mispairing alters the rate and position of AsCas12a cleavage	<u>ID 7</u> Fausta Michnevičiūt EMBL Partnership .
	Functionalized Lipid Nanoparticles for Hepatic Delivery of RNA- therapeutics	<u>ID 7</u> Simon Papa University of Cop
	Enhancing CRISPR-Cas9-Mediated Homology-Directed Repair Using Modified sgRNAs	<u>ID 7</u> Eyemen Khe University of Trent
	Enhancing the precision and fidelity of CRISPR/Cas9 genome editing through chemical inhibition of end-joining repair pathways	<u>ID 7</u> Sandra Wimberge Genome Engineerin.
	Design of highly efficient sgRNA libraries through comprehensive feature analysis	<u>ID 7</u> Lukas Badertsche Myllia Biotechnolog
	Image-enabled cell sorting and transcriptomic profiling to identify cellular phenotype-transcriptome relationships	<u>ID 6</u> Maria Gieble Emerging Innovati.
	Development of molecular biology assays to measure precision and fidelity of gene insertions	<u>ID 6</u> Karl Agge Novo Nordis
	Evaluation of CRISPR off-targets in single cells reveals previously unidentified off-targets and gives mechanistic insights in relation to cellular chromatin and gene expression state	ID 6 Alexandra Madse Genome Engineerin.

Detection of sgRNA via SHERLOCK for CRISPR-related gene doping control purposes

A CRISPR-based approach using deadCas9-sgRNA to detect SARS- CoV-2	<u>ID 12</u> Mustapha Aouida Hamad Bin Khalifa
Revolutionizing CRISPR/Cas9 Gene Therapy with Targeted Chemical Modulation	<u>ID 14</u> Xinlai Cheng Buchmann institut
Polyplex-based CRISPR/Cas9 In Vivo Mutagenesis for Efficient Lung Cancer Modeling in Rodents	<u>ID 15</u> Mirentxu Santos * Biomedical Innova
Sequence specific depletion of cancer cells using the dsDNA collateral activity of a novel CRISPR/Cas nuclease	<u>ID 16</u> Paul Scholz Akribion Genomics
CRISPR-Mediated Inactivation of Faah and Magl in Peripheral Nociceptive Neurons: Towards Modulating Endocannabinoid Levels for Pain Control.	<u>ID 17</u> Diana de Prado- Verdún Unidad de Innovac
Studying the role of TRIM28 and transposable elements dysregulation in neurodevelopmental disorders using CRISPRed in vitro models	<u>ID 18</u> Laura Castilla- Vallmanya Laboratory of Mol
Universal CAST-Seq: Off-target detection of CRISPR nucleases and base editors	<u>ID 19</u> Masako Kaufmann Institute for Tra
Base editing mediated correction of severe $\beta 0$ thalassemia mutations.	<u>ID 20</u> Giulia Hardouin Imagine Institute
Conquering gene editing off-target effects with Gibco™ CTS™ HiFi Cas9 Protein	<u>ID 21</u> Roland Leathers Thermo Fisher Sci
Precision Gene Editing Corrects Phenotypic Effects of Dominant Keratin 6 Mutation in Pachyonychia Congenita Patient Cells.	ID 22 Angeles Mencía entro de Investig
Xential: universal toxin-based selection for precise genome engineering	<u>ID 23</u> Anastasiia Rulina AstraZeneca
A CRISPR-Cas9 double-hit strategy mitigates on-target aberrations and chromosomal translocations	<u>ID 24</u> Parinaz Zarghamian Institute for Tra
Genome-wide CRISPR activation and interference screens for decoding the molecular signature of induced pluripotent stem cell to T cell differentiation	<u>ID 25</u> Íñigo Lacunza DTU Health Tech
Standardization and harmonization of gene editing in human induced pluripotent stem cells: tagging of a lysosomal protein using CRISPR/Cas9	<u>ID 26</u> Magdalena Madej Cell & Gene Thera
CRISPR/Cas9-based pipeline to introduce custom edits in patient T cells	ID 27 Shiva Dahal-Koirala Centre for molecu
REMAX: Innovative REframing Strategies to MAXimize Dystrophin Restoration in DMD	ID 30 Fatwa Adikusuma University of Ade
CRIPSR screen identifies UBE2A loss-of-function to confer bortezomib sensitivity in diffuse large B-cell lymphoma	ID 31 Hanne Due Department of Hem
p38 MAPK fuels proliferation stress and DNA damage impairing the functionality of genetically engineered hematopoietic stem and progenitor cells	ID 33 Roberta Vacca Vita-Salute San R

	Dicine
CRISPR based transcriptional monitoring system	Muhammad Zuhaib Khan TUM
Studying the pre-existing CRISPR/Cas9 immunogenicity in Icelanding population and circumventing the immunogenicity barrier	ID 35 Rakesh Chandode Respiratory & Imm
Direct delivery of Cas9 or base editor protein and guide RNA complex enables genome editing in the retina	<u>ID 37</u> Juliette Pulman Sorbonne Universi
Generation of heterozygous and homozygous NF1 lines from human induced pluripotent stem cells using CRISPR/Cas9 to investigate bone defects associated to neurofibromatosis type 1	ID 38 Nathalie Holic INSERM U861, I-St
Engineering CRISPR nucleases for allele-specific editing of dominant genetic diseases	ID 39 Alessio Conci Center for Regene
A new CRISPR-mediated Apc Knockout Allele leads to Pyloric Gland Adenoma-like Gastric Polyps in the Mice with C57BL/6; FVB/N Mixed Background	ID 40 Aysen Gunel Ozcan Hacettepe University
CRISPR/dCas9 gene editing for overexpressing apolipoprotein AI, paraoxonase 1 and cholesterol transporter ABC1 in hepatocytes - a promising tool to obtain functional HDL	<u>ID 41</u> Laura Toma Lipidomics Depart
Mitigating the risk of T cell receptor mispairing in CRISPR-T cell receptor- engineered human T cells	<u>ID 42</u> Laura Stentoft Grand Technical Univers
He-RASE: a fast cellular model to screen CRISPR/Cas systems editing efficiency and specificity for dominant mutations	<u>ID 43</u> Alessandra Fabrizi University of Mod
	ID 44

Antigen-scaffold-mediated expansion of CRISPR-Cas9 knockin CAR-Kristoffer Haurum and TCR-T cells generates highly enriched, efficacious T-cell products Johansen T-cells and cance...

The epigenome as a novel player in CRISPR safety

ID 45

CRISPR News

Eve Stalker MRC Toxicology Unit

06:30 - 07:15	Optional Social Event: Morning Run in Copenhagen (co-organiser: Viv	lion)
08:00 - 09:25	<b>Choosing the Right Gene-Editing Approach.</b> <i>Facilitator: Antonio Casini PhD (Alia Therapeutics)</i> Workshop A	
08:00 - 09:25	<b>Navigating the CRISPR Delivery Landscape.</b> Facilitator: Alessandro Umbach PhD (Laboratory of Molecular Virology, University of Trento, It Stage 2	aly)
09:30 - 10:15	Panel Discussion: Standards and Regulations Main Stage Moderator: Rebecca Roberts, Ph.D., CRISPR Medicine News	
	<ul> <li>Samantha Maragh, Dr., Leader, Genome Editing Program, National Ins &amp; Technology (NIST), USA.</li> </ul>	titute of Standards
	<ul> <li>Attila Sebe, MD, PhD., Clinical Assessor Advanced Therapy Medicinal I Paul-Ehrlich-Institute, Federal Institute for Vaccines and Biomedicines, Haematology, Cell and Gene Therapy, Germany.</li> </ul>	. ,
	<ul> <li>Lotte Dahl Nissen, MSc, Ph.D., Non-clinical Assessor, Quality Assessm Trials, Danish Medicines Agency (DKMA), Denmark.</li> </ul>	
	<ul> <li>Veronika Jekerle, PhD., Head of Pharmaceutical Quality, Human Medic Medicines Agency (EMA).</li> </ul>	ines, European
10:15 - 11:35	<b>Oral Session: Tools/Delivery</b> Main Stage Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy)	
	10:15 - 10:35 Advancing CRISPR medicine by probing the detection, design, and delivery	<u>ID 147</u> Yonglun Luo Aarhus University
	10:35 - 10:55 Xdrop®: changing the approach to gene editing validation and single-cell functional assays	<u>ID 143</u> Sidsel Alsing Samplix
	10:55 - 11:15 Find-and-replace CRISPR Genome Editing HDR2.0: a Promising Therapeutic Strategy	<u>ID 129</u> Ayal Hendel Bar-Ilan University
	11:15 - 11:35 From Allogeneic CAR-T Cells to SMART-CART to Fight Solid Tumors	<u>ID 151</u> Julien Valton Cellectis
10:15 - 11:35	Oral Session: Safety Stage 2	

Chairs: Toni Cathomen (University of Freiburg) and Roberto Nitsch (AstraZeneca)

10:15 - 10:35 Deciphering Cas9 immunogenicity ID 140 Roberto Nitsch AstraZeneca

CRISPRMED24 Program - The First CRISPR Medicine Conference, Copenhagen Denmark, April 23-25, 2024 (virtual event April 22).

Base editors provoke non-predictable chromosomal translocations and off-larget editing         Manuel Institute of Simon           10:55 - 11:15 INDUCE-seq: Ensuring the safe development of cell and gene therapies by gene editing         Iteration in Broken String           11:15 - 11:35 Updated outcomes of variant detection and quantitation from the first NIST Genome Editing Consortium Interlab Study         Iteration in Simon           11:35 - 12:00         Coffee Break         Iteration in Stage 2           12:00 - 13:20         Oral Session: Functional Genomics Stage 2         Iteration in Christ: Manuel Kaulch (Goethe University Frankfurt) and Bernhard Schmierer (Karolinska Institute, Sweden)           12:00 - 13:20         Oral Session: Functional Genomics Stage 2         Iteration in Protein String 12:20 - 12:40         Iteration in Protein String 12:20 - 12:40           12:00 - 13:20         Terational genomics tools to dissect genetic networks of rejuvenation prime editing         Fion II Procession in the intervention in Protein Brown String 12:40 - 13:00           12:00 - 13:20         Oral Session: Pre-clinical/Clinical Trials Main Stage Christ: Akesia Cavazza (University College Landon) and Waseem Qasim (UCL Great Ormond Street Institute of Child Health)           12:00 - 13:20         In vivo correction of human phenylyketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.         Madelynn Whit University of metabolism.           12:20 - 12:20 In vivo correction of human phenylyketonuria variants via prime editing cystic fibrosis - validation in pat			
INDUCE-seq: Ensuring the safe development of cell and gene therapies       Simon Broken String         by gene editing       11:15 - 11:35         Updated outcomes of variant detection and quantitation from the first NIST Genome Editing Consortium Interlab Study       Samantha Mit National InterNist Genome Editing Consortium Interlab Study         11:35 - 12:00       Coffee Break         12:00 - 13:20       Oral Session: Functional Genomics Stage 2 Chairs: Manue Kaulich (Goethe University Frankfurt) and Bernhard Schmierer (Karolinska Institute, Sweder)         12:00 - 12:20       Functional genomics tools to dissect genetic networks of rejuvenation       Jin Alae         12:20 - 12:40       Eherps International frameworks of rejuvenation       Jin Hospital tor         12:20 - 13:00       Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools       Fional Energy Internation Goethe University of and base editing       Jin Stage Chairs: Alaesia Cavazza (University College London) and Waseem Casim (UCL Great Ormond Street Institute of Chief Freetin)       Madelym Whit University of and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Madelym Whit University of and base editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       Jin Vin Manual Ka CRISPR and human induced pluripotent stem cells - the magic duo for medical research       Jin Hailon for Ku Leuven Far         13:00 - 13:20       Stefanie M		Base editors provoke non-predictable chromosomal translocations and	ID 74 Manuel Rhiel 1 Institute for T
Updated outcomes of variant detection and quantitation from the first NIST Genome Editing Consortium Interlab Study         Samantha Mi National Inter National Inter Nat		INDUCE-seq: Ensuring the safe development of cell and gene therapies	ID 144 Simon Reed Broken String Bio
12:00 - 13:20       Oral Session: Functional Genomics Stage 2 Chairs: Manuel Kaulich (Goethe University Fankfurt) and Bernhard Schmierer (Karolinska Institute, Sweden)         12:00 - 12:20       III Functional genomics tools to dissect genetic networks of rejuvenation       III Autor         12:20 - 12:40       Zhenya IV Deciphering the code of cancer: A deep dive into variants with saturation prime editing       Zhenya IV Hospital for         12:40 - 13:00       Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools       Fiona III Manuel Ki Goethe University         13:00 - 13:20       III "PRCISR CRISPR: How prior knowledge can drive hit confidence in perturbation genomics."       Manuel Ki Goethe University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chairs: Alessis Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Chaird Cavazza (Uni		Updated outcomes of variant detection and quantitation from the first	<u>ID 141</u> Samantha Maragh National Institut
Stage 2 Cheirs: Manuel Kaulich (Goethe University Frankfurt) and Bernhard Schmierer (Karolinska Institute, Sweden)         12:00 - 12:20       In Functional genomics tools to dissect genetic networks of rejuvenation       In Alter Junctional genomics tools to dissect genetic networks of rejuvenation         12:20 - 12:40       Zhenya IV Deciphering the code of cancer: A deep dive into variants with saturation prime editing       In Prophal for         12:40 - 13:00       Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools       Fional E         13:00 - 13:20       Oral Session: Pre-clinical/Clinical Trials Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute o Child Health)       Madelynn Whit University of and base editing: A gateway to equitable treatment of inborn errors of metabolism.         12:00 - 12:20       In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Madelynn Whit University of and base editing: A gateway to equitable treatment of inborn errors of metabolism.       In Madelynn Whit University of and base editing: A gateway to equitable treatment of inborn errors of metabolism.       In Vuicewore Far Vuicewore Far University of and base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       In Vuicewore Far Vuicewore Far Vu	11:35 - 12:00	Coffee Break	
Functional genomics tools to dissect genetic networks of rejuvenation         Jint           12:20 - 12:40         I           Deciphering the code of cancer: A deep dive into variants with saturation prime editing         I2:40 - 13:00           12:40 - 13:00         Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools         Image: Tool of the second	12:00 - 13:20	Stage 2	stitute, Sweden)
Deciphering the code of cancer: A deep dive into variants with saturation prime editing       Zhenya IV         12:40 - 13:00       12:40 - 13:00         Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools       Fiona II         13:00 - 13:20       "PRCISR CRISPR: How prior knowledge can drive hit confidence in perturbation genomics."       Manuel K. Goethe University College London) and Waseem Qasim (UCL Great Ormond Street Institute or Child Heeliti)         12:00 - 13:20       Oral Session: Pre-clinical/Clinical Trials       Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute or Child Heeliti)         12:00 - 13:20       12:00 - 12:20       In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Madelynn Whit University of Base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       II         12:40 - 13:00       II       Pia Johan II       III         CRISPR and human induced pluripotent stem cells - the magic duo for medical research       Pia Johan III       Pia Johan III         13:00 - 13:20       Stefanie M			ID 136 Jin Chen Altos Labs
Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools       Fiona B         13:00 - 13:20       13:00 - 13:20       Manuel KG         "PRCISR CRISPR: How prior knowledge can drive hit confidence in perturbation genomics."       Manuel KG         12:00 - 13:20       Oral Session: Pre-clinical/Clinical Trials       Main Stage         Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute of Child Health)       Madelynn Whit         12:00 - 12:20       In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Marianne C         12:20 - 12:40       Marianne C       Marianne C         Base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       Marianne C         12:40 - 13:00       Il       Pia Johar         CRISPR and human induced pluripotent stem cells - the magic duo for medical research       Pia Johar         13:00 - 13:20       Stefanie M		Deciphering the code of cancer: A deep dive into variants with saturation	ID 159 Zhenya Ivakine Hospital for Sick
"PRCISR CRISPR: How prior knowledge can drive hit confidence in perturbation genomics."       Manuel R Goethe University College conditional confidence in perturbation genomics."         12:00 - 13:20       Oral Session: Pre-clinical/Clinical Trials         Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute of Child Health)         12:00 - 12:20       In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Madelynn Whit University of Base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       In vivo correction of human induced pluripotent stem cells - the magic duo for medical research         13:00 - 13:20       Stefanie M		Identification of novel oncology targets using a combination of functional	ID 133 Fiona Behan GSK
Main Stage       Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute of Child Health)         12:00 - 12:20       12:00 - 12:20         In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism.       Madelynn Whit University of 2000         12:20 - 12:40       12:20 - 12:40       Marianne C         Rase and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models       Marianne C         12:40 - 13:00       CRISPR and human induced pluripotent stem cells - the magic duo for medical research       In vivo fibrosis - 13:20		"PRCISR CRISPR: How prior knowledge can drive hit confidence in	ID 149 Manuel Kaulich Goethe University
In vivo correction of human phenylketonuria variants via prime editing and base editing: A gateway to equitable treatment of inborn errors of metabolism. 12:20 - 12:40 Base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models 12:40 - 13:00 CRISPR and human induced pluripotent stem cells - the magic duo for medical research 13:00 - 13:20 Madelynn Whit University of Marianne C KU Leuven Fac CFTR mutations causing CFTR mutations causing Marianne C KU Leuven Fac CFTR mutations causing CFTR mutatio	12:00 - 13:20	Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormol	nd Street Institute of
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CRISPR and human induced pluripotent stem cells - the magic duo for Pia Johar Lund Univ medical research 13:00 - 13:20		Base and prime editing strategies to re-write CFTR mutations causing	ID 138 Marianne Carlon KU Leuven Faculty
Stefanie M		CRISPR and human induced pluripotent stem cells - the magic duo for	<u>ID 139</u> Pia Johansson Lund University
Casy-mediated precise and template-free gene editing of a muscular Experimental		13:00 - 13:20 Cas9-mediated precise and template-free gene editing of a muscular	<u>ID 32</u> Stefanie Müthel Experimental and

	dystrophy founder mutation: From single editing and off-target analysis to double editing and clinical translation				
13:30 - 14:30	Lunch Break				
14:30 - 16:30	<b>Oral Session: Functional Genomics II</b> Stage 2 Chairs: Bernhard Schmierer (Karolinska Institute, Sweden) and Manuel Kaulich (Goethe University Frankfurt)				
	14:30 - 14:50 CRISPR functional genomics as a tool in drug discovery	ID 131 Bernhard Schmierer Karolinska Instit			
	14:50 - 15:10 With technology to biology: The single-cell functional genomics revolution	<u>ID 160</u> Daniel Schraivogel EMBL			
	15:10 - 15:30 Integrating RNA structure and attention mechanisms for accurate CRISPR-Cas12a system gRNA efficiency prediction	ID 108 Ziyi Sheng Center for non-co			
	15:30 - 15:50 Advancing Oncology Drug Discovery through Pooled CRISPR Screening	<u>ID 166</u> Nikhil Gupta Cancer Research H			
	15:50 - 16:10 Genome-wide CRISPR-Cas9 screen for the elucidation of novel mediators of cytotoxicity and cytokine production in natural killer cells	<u>ID 36</u> Nutsa Burduli Karolinska Instit			
	16:10 - 16:30 Flexible and Scalable Genetic Screens for Discovery and Characterization of Novel Therapeutic Targets	<u>ID 167</u> Paul Diehl Cellecta			
14:30 - 16:30	<b>Oral Session: Safety II</b> Main Stage Chairs: Toni Cathomen (University of Freiburg, Germany) and Roberto Nitsch (AstraZeneca	a)			
	14:30 - 14:50 Pharmacological Interventions to Enhance Genome Editing Precision	<u>ID 165</u> Marcello Maresca AstraZeneca			
	14:50 - 15:10 Next-Level Insights: High-Resolution Analysis of Chromosomal Aberrations with NHEJ Inhibition	<u>ID 46</u> Giandomenico Turchiano UCL			
	15:10 - 15:30 End-to-End Tools for Interrogation of CRISPR-Cas Associated Genotoxicity	<u>ID 127</u> Ashley Jacobi Integrated DNA Te			
	15:30 - 15:50 Investigating the interplay between CRISPR-induced double-strand breaks and recombinant AAV Integration in vivo	<u>ID 107</u> Laura Torella DNA & RNA Medicin			
	15:50 - 16:10 Karyotyping and FISH assays as tools to evaluate the genetic stability of Cell therapy products - A decade of GMP experience	<u>ID 169</u> Vasileios Georgakakos Clean Cells			

	16:10 - 16:30 Evaluation of CRISPR off-targets in single cells reveals previously unidentified off-targets and gives mechanistic insights in relation to cellular chromatin and gene expression state	ID 67 Alexandra Madsen Genome Engineerin			
16:30 - 16:45	Coffee Break				
16:45 - 18:25	<b>Oral Session: Pre-clinical/ Clinical Trials II</b> Main Stage Chairs: Waseem Qasim (UCL Great Ormond Street Institute of Child Health) and Alessia ( London)	Cavazza (University College			
	16:45 - 17:05 Realizing the Promise of CRISPR Therapeutics	<u>ID 164</u> Laura Sepp-Lorenzino Intellia Therapeu			
	17:05 - 17:25 The leads code and its relevance to eliminat development of ODIODD	<u>ID 8</u> Kelly Banas			
	The Indel Code and its relevance to clinical development of CRISPR- directed gene editing in cancer	ChristianaCare Ge			
	17:25 - 17:45 Implementation of gene editing to correct hematopoietic stem cells from Fanconi anemia patients	ID 153 Paula Rio CIEMAT			
	17:45 - 18:05 CRISPR/Cas9-mediated gene editing delivered by a single AAV vector inhibits viral reactivation of HSV-1 in a latent rabbit keratitis model	ID 81 Kevin Luk Excision BioThera			
	18:05 - 18:25 Enabling GMP Production of sgRNA for CRISPR-based Cell and Gene Therapies	<u>ID 155</u> Kevin Holden Synthego			
16:45 - 18:25	<b>Oral Session: Tools/Delivery II</b> Stage 2 Chairs: Anna Cereseto (University of Trento, Italy) and Marcello Maresca (AstraZeneca)				
	16:45 - 17:05 FiCAT gene writing platform for advanced therapies	<u>ID 128</u> Avencia Sanchez- Mejías Integra Therapeutics			
	17:05 - 17:25 Computational CRISPR/Cas9 gRNA design	<u>ID 135</u> Jan Gorodkin University of Cop			
	17:25 - 17:45 Engineered CRISPR Technologies to Improve Genome Editing	<u>ID 130</u> Ben Kleinstiver Massachusetts Gen			
	17:45 - 18:05 Epigenome editing as a novel and safe strategy to control multiple immune checkpoints expression in CAR T cells	ID 90 Maria Silvia Roman Azcona Institute for Tra			
	18:05 - 18:25 Base editing based on Cas12a variants	<u>ID 158</u> Stefano Stella Ensoma			
19:30 - 22:00	Conference Dinner Conference Venue				

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ID 157 Andre Cohnen

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ID 134 Howard Wu

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ID 132 Fan Zhou

<u>ID 150</u>

Alessia Cavazza

University Colleg...

GenScript

Maaike De Cock

Thomas W. Skov

Department of Bio...

Full Circles Ther...

Paula M. Cevaal

Department of Inf...

**Ghent University** 

## Thursday, 25 April 2024 (times in CEST) 08:00 - 09:25 The Road to Clinical Translation. Facilitator: Members of Simone Spuler's group, Charité Universistätsmedizin Berlin / Max Delbrück Center Stage 2 08:00 - 09:25 Pre-Clinical Safety Analyses. Facilitators: Carla F. García PhD, Julia Klermund PhD and Manuel Rhiel PhD (Uni. Medical Center Freiburg) Workshop A 09:30 - 11:30 **Oral Session: Tools/Delivery III** Stage 2 Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy) 09:30 - 09:50 CD4 T cell targeting lipid nanoparticles in the search for a genetic HIV cure. 09:50 - 10:10 Improved synthetic RNA-guided nucleases for human therapeutic use 10:10 - 10:30 Efficient rAAV6-mediated HDR with low toxicity as a therapy for GATA2 deficiency through allele-specific gene correction in hematopoietic stem cells 10:30 - 10:50 Less is More: Efficient Novel Non-Viral Immune Cell Engineering With Precise Genomic Integration 10:50 - 11:10 Lipid nanoparticle delivery of dCas9-SAM to primary T-cells: towards a CRISPR activation-based therapeutic to cure HIV 11:10 - 11:30 Novel DNA Payloads and One-Stop CRISPR Toolbox Accelerate Non-Viral Gene Editing Therapeutics Development 09:30 - 11:30 **Oral Session: Pre-clinical/Clinical Trials III** Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute of Child Health) 09:30 - 09:50 Preclinical development of gene editing therapies 09:50 - 10:10

ID 156 Raoul Hennig BioSpring

ID 80 Xiaoxia Cui Washington Univer...

> ID 126 Antonio Casini Alia Therapeutics

A novel portfolio approach to CRISPR-based gene therapies with tailored advantages for human therapeutics

Efficient large knockins in mice and cells validated using an all-in-one,

Charting New Horizons in guide RNA Manufacturing

multiplexed long-read sequencing assay

10:10 - 10:30

10:30 - 10:50

CRISPRMED24 Program - The First CRISPR Medicine Conference, Copenhagen Denmark, April 23-25, 2024 (virtual event April 22).

14:00 - 14:15	Closing address by CMN Main Stage	
13:05 - 14:00	Lunch Break	
	12:45 - 13:05 Revolutionizing CRISPR/Cas9 Gene Therapy with Targeted Chemical Modulation	<u>ID 14</u> Xinlai Cheng Buchmann institut
	12:25 - 12:45 CRISPR associated substrate-linked directed evolution (CaSLiDE) for evolving highly efficient and specific miniature CRISPR-Cas systems	<u>ID 99</u> Duran Sürün Medical Faculty a
	12:05 - 12:25 Software Tools to Enable CRISPR Therapeutics Discovery & Design	<u>ID 179</u> Pawan Patel Benchling
	11:45 - 12:05 Pioneering Precision Medicines Using Microbial CRISPR Gene Therapy	ID 185 Jonas Hink SNIPR Biome
11:45 - 13:05	<b>Oral Session: Tools/Delivery IV</b> Stage 2 Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy)	
	13:05 - 13:25 Exploiting Targeted Epigenome Editing for Therapeutic Applications	<u>ID 124</u> Angelo Lombardo San Raffaele Tele
	12:45 - 13:05 Genetic and transcriptional engineering of primary human blood cells	ID 168 Rasmus O. Bak Aarhus University
	12:25 - 12:45 Genome edited therapeutic T cells	ID 170 Waseem Qasim University Colleg
	12:05 - 12:25 Base editing mediated correction of severe β0 thalassemia mutations.	<u>ID 20</u> Giulia Hardouin Imagine Institute
	11:45 - 12:05 Project Delta Force - Upregulating delta globin as a new avenue to treat hemoglobinopathies	<u>ID 162</u> Jan Nelis Ariya Bio
11:45 - 13:25	<b>Oral Session: Pre-clinical/Clinical Trials IV</b> Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormon Child Health)	d Street Institute of
11:30 - 11:45	Coffee Break	
	11:10 - 11:30 Reinventing Cardiovascular Disease Treatment with Single-Course Gene Editing Medicines	ID 154 Andrew Bellinger Verve Therapeutics
	10:50 - 11:10 Precision targeting tumor cells using cancer-specific genetic alterations with CRISPR/Cas	ID 92 Taejoon Kwon Ulsan National In

14:00 - 17:00 **Optional Social Event: Guided boat trip in the Copenhagen canals** Copenhagen

> CRISPRMED25 Stay updated <u>here</u>