Monday, 22 April 2024

15:00 - 16:20	Virtual Event - Selected Presentations	
	Virtual Selected Presentations	
	15:00 - 15:20	<u>ID 175</u>
	Epigenome Editing for the Effective Treatment of HBV	Brian Cosgrove
	1.5	Tune Therapeutics
	15:20 - 15:40	<u>ID 176</u>
	Advances and Challenges of Next-Generation CRISPR Gene-Editing Technology for Drug	Alvin Luk HuidaGene Therape
	Development	Huldagene merape
	15:40 - 16:00	ID 181
	EMA's Experience & Support to the Development of Medicinal Products Using Genome	Veronika Jekerle
	Editin	European Medicine
	16:00 - 16:20	<u>ID 182</u>
	A New Approach to Tackle Cancer Using Programmable Cytotoxic Nucleases	Michael Krohn
16:20 - 16:30	Virtual Coffee Break	Akribion Genomics
10.20 - 10.30	Vii tuai Conee Break	
16:30 - 18:00	Virtual Event - Poster Session	
	Virtual Poster Session	
	Polyplex-based CRISPR/Cas9 In Vivo Mutagenesis for Efficient Lung Cancer Modeling in	<u>ID 15</u>
	Rodents	Mirentxu Santos *
		Biomedical Innova ID 17
	CRISPR-Mediated Inactivation of Faah and Magl in Peripheral Nociceptive Neurons:	Diana de Prado-Verdún
	Towards Modulating Endocannabinoid Levels for Pain Control.	Unidad de Innovac
	Disruption of Epidermal Growth Factor Binding Site by Sequential Knock in	<u>ID 29</u>
	CRISPR/Cas9 Genome Editing of Epidermal Growth Factor Receptor and Evaluation of	Jelena Popovio
	Introduced Mutations in Cervical Cancer Cells	Radiation Oncolog
	Duradisting Off Toward Astivity in the CDICDD/Codd Costage Lieins Multilever Development	<u>ID 53</u>
	Predicting Off-Target Activity in the CRISPR/Cpf1 System Using Multilayer Perceptron	Pragya Kesarwani Regional Centre f
		ID 72
	Enhancing CRISPR-Cas9-Mediated Homology-Directed Repair Using Modified sgRNAs	Eyemen Kheir
		University of Trento
		<u>ID 78</u>
	Prenatal In Vivo Base Editing for the Treatment of Krabbe Disease	Asma Naseem
		1Department of In
	EDSpliCE: A Novel Gene Editing Platform Holding Therapeutic Potential for Splicing	<u>ID 83</u> Pietro De Angeli
	Modulation in Inherited Retinal Disorders	Institute for Oph
		ID 88
	CRISPR/Cas9 Based Knock-In/Knock-Out of RAG2 Gene in Jurkat Cells as Model for	Zeinab Yousefi-
	Genome Editing Screening	Najafabadi
		Department of Med

Tuesday, 23 April 2 14:00 - 14:15	Welcome and opening address by CMN	
	Main Stage	
14:15 - 15:00	Keynote Lecture - Precision Genetic Engineering of Hematopoiesis by Gene Editing	Tools
	Main Stage	
		<u>ID 137</u>
	Precision Genetic Engineering of Hematopoiesis by Gene Editing Tools	Luigi Naldin San Raffaele Tele
15:00 - 15:15	Coffee Break	
	Main Stage	
15:15 - 17:45	Selected talks - one featured presentation from each track	
	Main Stage	
	Chairs: Alessia Cavazza (University College London) and Marcello Maresca (AstraZeneca)	
	15:15 - 15:40	<u>ID 152</u>
	Transforming Gene Therapy From the Few to the Many	Karina Thorr Novo Nordisl
	15:40 - 16:05	<u>ID 125</u>
	Identification and Evolution of Novel CRISPR-Cas9 Systems From the Human	Anna Cereseto
	Microbiome	Department of Cel
	16:05 - 16:30	ID 163
		Karim Benabdellah E
	Connecting Innovators: CA21113 2nd Year Journey in Fostering Collaborative Networks	Khlanj
	for Breakthroughs in Genome Editing to Treat Human Diseases (GenE-Humdi COST	GENYO, Centre for
	action)	
	16:30 - 16:55	<u>ID 145</u> Simone Spulei
	Precisely Edited Primary Human Muscle Stem Cells as an ATMP in Muscular	Charité Universit
	Dystrophies	
	16:55 - 17:20	ID 146
	New Insights Into On- And Off-Target Effects of Genome Editing Tools	Toni Cathomer University Freiburg
	17:20 - 17:45	ID 142
	CRISPR-Based Functional Genomic Characterization of Mechanisms of Action of	Seung Wook Yang
	Degraders for Targeted Protein Degradation	AMGEN
17:45 - 20:00	Poster Session / Networking Session with Snack	
17.43 - 20.00	Poster Area	
17:50 - 18:30	Poster Session A	
17.50 - 16.50	Poster Session A	
	EDSpliCE: A Novel Gene Editing Platform Holding Therapeutic Potential for Splicing	<u>ID 83</u>
	Modulation in Inherited Retinal Disorders	Pietro De Angel Institute for Oph
		ID 84
	Gene editing in hematopoietic stem cells by delivery of CRISPR-based genome editing tool kits in lentivirus-derived nanoparticles (LVNPs)	Sofie Anderser
	tool kits in lentuvii us-ueriveu hanoparticles (Evivi s)	Department of Bio
	Exploring the functional impact of lncRNAs through CRISPRi screens in CRC models	<u>ID 86</u> Ante Mihaljevio
	with KRAS and BRAF mutational background	Department for Bi
		ID 87
	Prime editing-mediated correction of the FANCA gene in primary patient cells	Karolina Skvarova Kramarzova
		Childhood Leukaem
	Highly efficient gene editing in human primary T cells using novel CRISPR effectors	<u>ID 91</u>
	from the human microbiome	Antonio Carusillo
		Alia Therapeutics ID 94
	Implementation of TRuC T cells for the treatment of prostate cancer	الم
		Institute for Tra

Engineered baculoviral protein and DNA delivery platform for large DNA cargo integration and enhanced gene editing in human cells	<u>ID 95</u> 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	ID 98 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	ID 102 Cosimo Ducani Moligo Technologies
Nomination of Genome-Wide CRISPR-Cas9 Cleavage Activity using rhAmp Technology	ID 103 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	ID 105 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.	ID 109 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	ID 112 Wenhao Gao Center for non-co
Optimized shRNAmir screens unveil cancer vulnerabilities in the essential gene space.	<u>ID 113</u> 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	<u>ID 115</u> 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	<u>ID 118</u> 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	ID 122 Jure Bohing Department of Syn

	In Vivo Correction of a Genetically Humanized Fanconi Anemia Mouse Model Using Digital Editing Technologies	Colette B. Rogers
	Less is More: Efficient Novel Non-Viral Immune Cell Engineering With Precise Genomic Integration	Department of Ped ID 134 Howard Wu Full Circles Ther
18:30 - 19:15	Poster Session B	ruii Circles Triet
	Development and validation of a novel PD-1/PD-L1 and PD-L2 co-inhibition assay for CRISPR-screening in T cells	<u>ID 47</u> Beatrice Scapolo T-cells and cance
	Novel characterization assays enable efficient GMP manufacturing of CRISPR RNP complexes	<u>ID 49</u> Thomas Lynch Aldevror
	Gene-edited primary muscle stem cells rescue dysferlin-deficient muscular dystrophy	<u>ID 5(</u> Helena Escoba Charité - Univers
	Revolutionizing CRISPR Therapies: A Scalable Manufacturing Platform for eVLPs production	<u>ID 52</u> Lúcia Santo: iBET, Instituto d
	Predicting Off-Target Activity in the CRISPR/Cpf1 System Using Multilayer Perceptron	<u>ID 53</u> Pragya Kesarwan Regional Centre f
	CRISPRBITS- Empowering Health Precision	<u>ID 54</u> Reety Arora CRISPRBITS
	Development of a lipidoid library for Cas9-RNP-delivery	<u>ID 55</u> Zian Xue University of Vienna
	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 56</u> Michael Crowthe National Center f
	Base editing of hematopoietic stem cells restores immune function in a mouse model of familial hemophagocytic lymphohistiocytosis	<u>ID 57</u> Lei Le Institute for Tra
	Development of a CRISPR-based epigenome editing platform to enhance the long-term engraftment of ex-vivo genetically modified HSPCs	ID 58 Federica Zinghiring University Colleg
	Improved CRISPR/Cas9 Off-target Assessment based on Guide RNA Binding Energy	<u>ID 59</u> Dhvani Vora Center for non-Co
	Genome-wide CRISPR-Cas9 knockout screen identifies DNA damage response pathways and BTK as essential for cisplatin response in diffuse large B-cell lymphoma	<u>ID 6</u> Issa Ismail Issa Department of Hem.
	Enhanced CRISPR base editing design from data generation and deep learning	<u>ID 62</u> Ying Sur Center for non-co
	Genome-wide CRISPR screening in postmitotic hepatocytes of murine liver	<u>ID 63</u> Joonsun Lee Institute of Mole
	MyoPax: the vanguard in cell and gene therapies for muscle disorders	<u>ID 64</u> Eric Metzler-Bräuchle MyoPax GmbH
	Efficient single-cell cloning and culture of hiPSCs in small-scale fluid-walled cell culture chambers	<u>ID 6</u> Narasimha Telugu MaxDelbruck cente
	CRISPRon/off: Combined CRISPR/Cas9 on- and off-target design	<u>ID 66</u> Christian Anthor University of Cop
	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 Madser Genome Engineerin
	Development of molecular biology assays to measure precision and fidelity of gene insertions	<u>ID 68</u> Karl Agge Novo Nordisl

	Image-enabled cell sorting and transcriptomic profiling to identify cellular phenotype-transcriptome relationships	<u>ID 69</u> Maria Giebler Emerging Innovati
	Design of highly efficient sgRNA libraries through comprehensive feature analysis	ID 70 Lukas Badertscher Myllia Biotechnology
	Enhancing the precision and fidelity of CRISPR/Cas9 genome editing through chemical inhibition of end-joining repair pathways	<u>ID 71</u> Sandra Wimberger Genome Engineerin
	Enhancing CRISPR-Cas9-Mediated Homology-Directed Repair Using Modified sgRNAs	ID 72 Eyemen Kheir University of Trento
	Functionalized Lipid Nanoparticles for Hepatic Delivery of RNA-therapeutics	ID 73 Simon Papa University of Cop
	Off-targets for good: Mispairing alters the rate and position of AsCas12a cleavage	<u>ID 75</u> Fausta Michnevičiūtė EMBL Partnership
	Generation of an experimental model for whole genome CRISPR screening to study hypoxia-induced drivers of chemotherapy resistance in muscle invasive bladder cancer	ID 76 Danielle Smith Univeristy of Man
	ePsCas9- a CRISPR tool for in vivo therapeutic genome editing at AstraZeneca	ID 77 Aikaterini Emmanouilidi Genome Engineerin
	Unraveling genome integrity and safety of gene engineered hematopoietic stem and progenitor cells for the treatment of RAG1 deficiency	<u>ID 79</u> Chiara Brandas San Raffaele-Tele
	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
	Prime editing for correction of GATA2 deficiency in human CD34+ hematopoietic stem cells	<u>ID 82</u> Jonas H. Wolfl Department of Bio
	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.
	Software Tools to Enable CRISPR Therapeutics Discovery & Design	<u>ID 179</u> Pawan Patel Benchling
19:15 - 20:00	Poster Session C	
	Detection of sgRNA via SHERLOCK for CRISPR-related gene doping control purposes	<u>ID 10</u> Alina Paßreiter Center for Preven
	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	ID 15 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	I <u>D 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 β0 thalassemia mutations.	<u>ID 20</u> Giulia Hardouin Imagine Institute
	Conquering gene editing off-target effects with Gibco™ CTS™ HiFi Cas9 Protein	ID 21 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
	A CRISPR-Cas9 double-hit strategy mitigates on-target aberrations and chromosomal translocations	AstraZeneca <u>ID 24</u> Parinaz Zarghamian
	Genome-wide CRISPR activation and interference screens for decoding the molecular signature of induced pluripotent stem cell to T cell differentiation	Institute for Tra ID 25 Íñigo Lacunza
	Standardization and harmonization of gene editing in human induced pluripotent stem cells: tagging of a lysosomal protein using CRISPR/Cas9	DTU Health Tech <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
	Using MD Simulations to Design More Efficient Lipid Nanoparticles	ID 28 Florian A. Mann Chemical & Pharma
	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	I <u>D 33</u> Roberta Vacca Vita-Salute San R
	CRISPR based transcriptional monitoring system	<u>ID 34</u> 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	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	<u>ID 38</u> 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 Al, paraoxonase 1 and cholesterol transporter ABC1 in hepatocytes - a promising tool to obtain functional HDL	ID 41 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 Fabriz University of Mod
	Antigen-scaffold-mediated expansion of CRISPR-Cas9 knockin CAR- and TCR-T cells generates highly enriched, efficacious T-cell products	<u>ID 44</u> Kristoffer Haurum
·		

	Johansen
	T-cells and cance
	<u>ID 45</u>
The epigenome as a novel player in CRISPR safety	Eve Stalker
	MRC Toxicology Unit
	ID 46
MEGA dPCR: A Comprehensive Analysis Tool for Unbiased Quantification of Genome	Giandomenico
Integrity and Mutation Dynamics in Clinical Gene Editing	Turchiano
	UCL

Wednesday, 24 April 2024

Wednesday, 24 Ap 06:30 - 07:00	Optional Social Event: Morning Fun Run in Copenhagen (co-organised with Vivlion)	
08:00 - 09:25	Choosing the Right Gene-Editing Approach. Facilitator: Antonio Casini PhD (Alia Ther Workshop A	apeutics)
08:00 - 09:25	Navigating the CRISPR Delivery Landscape. Facilitator: Alessandro Umbach PhD (Lab	oratory of Molecular
00.00 03.23	Virology, University of Trento, Italy)	or acory or more carar
	Workshop B	
09:30 - 10:15	Panel Discussion: Standards and Regulations	
03.30 10.13	Main Stage	
	 Samantha Maragh, Dr., Leader, Genome Editing Program, National Institute of St (NIST), USA. Attila Sebe, MD, PhD., Clinical Assessor Advanced Therapy Medicinal Products (A) 	
	Institute, Federal Institute for Vaccines and Biomedicines, Division of Haematology, Cell and Gene Therapy, Germany.	
	 Lotte Dahl Nissen, MSc, Ph.D., Non-clinical Assessor, Quality Assessment & Clinic Medicines Agency (DKMA), Denmark. 	ai IIIais, Dailisii
	 Veronika Jekerle, PhD., Head of Pharmaceutical Quality, Human Medicines, Europ Agency (EMA). 	oean Medicines
10:15 - 11:35	Oral Session: Tools/Delivery	
10.15 11.55	Stage 2	
	Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy)	
	10:15 - 10:35	<u>ID 14</u>
		Yonglun Lu
	Advancing CRISPR medicine by probing the detection, design, and delivery	Aarhus University
	10:35 - 10:55	<u>ID 17′</u>
	Translating in vivo gene editing to the Microbiome	Xavier Duporte Eligo Bioscience
	10:55 - 11:15	ID 129
	Find-and-replace CRISPR Genome Editing HDR2.0: a Promising Therapeutic Strategy	Ayal Hende
	11:15 - 11:35	Bar-llan University ID 151
	From Allogeneic CAR-T Cells to SMART-CART to Fight Solid Tumors	Julien Valtor Cellectis
10:15 - 11:35	Oral Session: Safety	Concess
	Main Stage	
	Chairs: Toni Cathomen (University of Freiburg) and Roberto Nitsch (AstraZeneca)	
	10:15 - 10:35	<u>ID 140</u> Roberto Nitsch
	Deciphering Cas9 immunogenicity	AstraZeneca
	10:35 - 10:55	ID 74
	Base editors provoke non-predictable chromosomal translocations and off-target	Manuel Rhie 1 Institute for T
	editing	i institute for 1
	10:55 - 11:15	<u>ID 14</u> 4
	INDUCE-seq: Ensuring the safe development of cell and gene therapies by gene editing	Simon Reed
	11:15 - 11:35	Broken String Bio ID 141
	Updated outcomes of variant detection and quantitation from the first NIST Genome	Samantha Maragh
	Editing Consortium Interlab Study	National Institut
11:35 - 12:00	Coffee Break	
12:00 - 13:20	Oral Session: Functional Genomics	
	Stage 2	
	Chairs: Manuel Kaulich (Goethe University Freiburg) and Bernhard Schmierer (Karolinska Institu	te, Sweden)
	12:00 - 12:20	ID 136
	Functional genomics tools to dissect genetic networks of rejuvenation	Jin Cher Altos Labs
		Altos Lab:

	12:20 - 12:40 Deciphering the code of cancer: A deep dive into variants with saturation prime editing	<u>ID 159</u> Zhenya lvakine Hospital for Sick
	12:40 - 13:00 Identification of novel oncology targets using a combination of functional genomics approaches & machine learning tools	<u>ID 133</u> Fiona Behan GSK
	13:00 - 13:20 "PRCISR CRISPR: How prior knowledge can drive hit confidence in perturbation genomics."	<u>ID 149</u> Manuel Kaulich Goethe University
12:00 - 13:20	Oral Session: Pre-clinical/Clinical Trials	
	Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond S Health)	itreet Institute of Child
	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.	<u>ID 9</u> Madelynn Whittaker University of Pen
	12:20 - 12:40 Base and prime editing strategies to re-write CFTR mutations causing cystic fibrosis - validation in patient derived cell models	<u>ID 138</u> Marianne Carlon KU Leuven Faculty
	12:40 - 13:00 CRISPR and human induced pluripotent stem cells - the magic duo for medical research	<u>ID 139</u> Pia Johansson Lund University
	13:00 - 13:20 Cas9-mediated precise and template-free gene editing of a muscular dystrophy founder mutation: From single editing and off-target analysis to double editing and clinical translation	<u>ID 32</u> Stefanie Müthel Experimental and
13:30 - 14:30	Lunch Break	
14:30 - 16:30	Oral Session: Functional Genomics II Stage 2 Chairs: Bernhard Schmierer (Karolinska Institute, Sweden) and Manuel Kaulich (Goethe Univer	
	14:30 - 14:50 CRISPR functional genomics as a tool in drug discovery	<u>ID 131</u> 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	<u>ID 108</u> 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 Burdul 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	Oral Session: Safety II Main Stage Chairs: Toni Cathomen (University of Freiburg, Germany) and Roberto Nitsch (AstraZeneca)	

	14:30 - 14:50	ID 165
	Pharmacological Interventions to Enhance Genome Editing Precision	المان Marcello Maresca
		AstraZeneca ID 117
	14:50 - 15:10	<u>ID 117</u> Saumyaa Saumyaa
	Novel tools for Gene and Cell Therapy safety: Optical long-read genomics for detection	AstraZeneca
	and characterization of on- and off-target transgene integrations and off-target	
	structural variants	
	15:10 - 15:30	<u>ID 127</u> Ashley Jacob
	End-to-End Tools for Interrogation of CRISPR-Cas Associated Genotoxicity	Integrated DNA Te
	15:30 - 15:50	<u>ID 107</u>
	Investigating the interplay between CRISPR-induced double-strand breaks and	Laura Torella DNA & RNA Medicin
	recombinant AAV Integration in vivo	2.0.00.00.00.00.00.00.00.00.00.00.00.00.
	15:50 - 16:10	<u>ID 169</u>
	Karyotyping and FISH assays as tools to evaluate the genetic stability of Cell therapy	Vasileios Georgakakos Clean Cells
	products - A decade of GMP experience	Clean Cens
_	16:10 - 16:30	<u>ID 67</u>
	Evaluation of CRISPR off-targets in single cells reveals previously unidentified off-	Alexandra Madsen
	targets and gives mechanistic insights in relation to cellular chromatin and gene	Genome Engineerin
	expression state	
16:30 - 16:45	Coffee Break	
16:45 - 18:25	Oral Session: Pre-clinical/ Clinical Trials II	
	Main Stage	
	Chairs: Waseem Qasim (UCL Great Ormond Street Institute of Child Health) and Alessia Cavaz	zza (University College
	London)	, , , ,
	16:45 - 17:05	<u>ID 164</u>
	Realizing the Promise of CRISPR Therapeutics	Laura Sepp-Lorenzino Intellia Therapeu
	17:05 - 17:25	Intella Therapeu
	The Indel Code and its relevance to clinical development of CRISPR-directed gene	Kelly Banas
	editing in cancer	ChristianaCare Ge
	17:25 - 17:45	<u>ID 153</u>
	Implementation of gene editing to correct hematopoietic stem cells from Fanconi	Paula Rio
	anemia patients	CIEMAT
	17:45 - 18:05	ID 81
	CRISPR/Cas9-mediated gene editing delivered by a single AAV vector inhibits viral	Kevin Luk
	reactivation of HSV-1 in a latent rabbit keratitis model	Excision BioThera
	18:05 - 18:25	ID 155
	Enabling GMP Production of sgRNA for CRISPR-based Cell and Gene Therapies	Kevin Holden
16:45 - 18:25	Oral Session: Tools/Delivery II	Synthego
	Stage 2	
	Chairs: Anna Cereseto (University of Trento, Italy) and Marcello Maresca (AstraZeneca)	
	16:45 - 17:05	<u>ID 128</u>
	FiCAT gene writing platform for advanced therapies	Avencia Sanchez-Mejías
	17:05 - 17:25	Integra Therapeutics ID 135
	Computational CRISPR/Cas9 gRNA design	Jan Gorodkir
		University of Cop
	17:25 - 17:45	<u>ID 130</u> Ben Kleinstiver
	Engineered CRISPR Technologies to Improve Genome Editing	Massachusetts Gen

	17:45 - 18:05	<u>ID 90</u>
	Epigenome editing as a novel and safe strategy to control multiple immune	Maria Silvia Roman
	1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1	Azcona
	checkpoints expression in CAR T cells	Institute for Tra
	18:05 - 18:25	<u>ID 158</u>
	Base editing based on Cas12a variants	Stefano Stella
	base editing based on easiza variants	Ensoma
19:30 - 23:00	Conference Dinner	
	Conference Venue	

Thursday, 25 April 2024

Thursday, 25 April 08:00 - 09:25	The Road to Clinical Translation. Faciliator: Members of Simone Spuler's group, Char	rité	
	Universistätsmedizin Berlin / Max Delbrück Center		
	Workshop B		
08:00 - 09:25	Pre-Clinical Safety Analyses. Facilitators: Carla F. García PhD, Julia Klermund PhD an	d Manuel Rhiel PhD	
	(Uni. Medical Center Freiburg)		
	Workshop A		
09:30 - 11:30	Oral Session: Tools/Delivery III		
	Main Stage		
	Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy)		
	09:30 - 09:50	<u>ID 13</u>	
	CD4 T cell targeting lipid nanoparticles in the search for a genetic HIV cure.	Maaike De Cock Ghent University	
	09:50 - 10:10	ID 157	
	Improved synthetic RNA-guided nucleases for human therapeutic use	Andre Cohner Baye	
	10:10 - 10:30	ID 85	
	Efficient rAAV6-mediated HDR with low toxicity as a therapy for GATA2 deficiency	Thomas W. Skov	
	through allele-specific gene correction in hematopoietic stem cells	Department of Bio	
	10:30 - 10:50	ID 134	
l	Less is More: Efficient Novel Non-Viral Immune Cell Engineering With Precise Genomic	Howard Wu	
	Integration	Full Circles Ther	
		ID 51	
	10:50 - 11:10	Paula M. Cevaa	
	Lipid nanoparticle delivery of dCas9-SAM to primary T-cells: towards a CRISPR	Department of Inf	
	activation-based therapeutic to cure HIV		
	11:10 - 11:30	<u>ID 132</u> Fan Zhou	
	Novel DNA Payloads and One-Stop CRISPR Toolbox Accelerate Non-Viral Gene Editing	GenScrip	
	Therapeutics Development	•	
09:30 - 11:30	Oral Session: Pre-clinical/Clinical Trials III		
	Stage 2		
	Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond St	reet Institute of Child	
	Health)		
	09:30 - 09:50	<u>ID 150</u>	
	Preclinical development of gene editing therapies	Alessia Cavazza University Colleg	
	09:50 - 10:10	ID 156	
	Charting New Horizons in guide RNA Manufacturing	Raoul Hennig	
	10:10 - 10:30	BioSpring ID 80	
	Efficient large knockins in mice and cells validated using an all-in-one, multiplexed long-	Xiaoxia Cu	
	read sequencing assay	Washington Univer	
		ID 120	
	10:30 - 10:50	<u>ID 126</u> Antonio Casin	
	A novel portfolio approach to CRISPR-based gene therapies with tailored advantages	Alia Therapeutics	
	for human therapeutics		
	10:50 - 11:10	<u>ID 92</u> Taejoon Kwor	
	Precision targeting tumor cells using cancer-specific genetic alterations with	Ulsan National In	
	CRISPR/Cas		
	11:10 - 11:30	ID 154	
	Reinventing Cardiovascular Disease Treatment with Single-Course Gene Editing	Andrew Bellinge Verve Therapeutics	
	Medicines	verve merapeaties	

11:45 - 13:25	Oral Session: Pre-clinical/Clinical Trials IV	
	Main Stage Chairs: Alessia Cavazza (University College London) and Waseem Qasim (UCL Great Ormond Street Institute of Child	
	11:45 - 12:05	ID 163
		Project Delta Force - Upregulating delta globin as a new avenue to treat
	hemoglobinopathies	Ariya Bio
	12:05 - 12:25	ID 20
	Base editing mediated correction of severe β0 thalassemia mutations.	Giulia Hardouii Imagine Institute
	12:25 - 12:45	<u>ID 170</u>
	Genome edited therapeutic T cells	Waseem Qasin University Colleg
	12:45 - 13:05	ID 168
	Genetic and transcriptional engineering of primary human blood cells	Rasmus O. Ba
	13:05 - 13:25	Aarhus University
		Angelo Lombardo
	Exploiting Targeted Epigenome Editing for Therapeutic Applications	San Raffaele Tele
11:45 - 13:25	Oral Session: Tools/Delivery IV	
	Stage 2	
	Chairs: Marcello Maresca (AstraZeneca) and Anna Cereseto (University of Trento, Italy)	
	11:45 - 12:05	<u>ID 161</u>
	Pioneering precision medicines using microbial CRISPR gene therapy	Christian Groendah SNIPR Biome
	12:05 - 12:25	ID 179
	Software Tools to Enable CRISPR Therapeutics Discovery & Design	Pawan Pate Benchling
	12:25 - 12:45	ID 99
	CRISPR associated substrate-linked directed evolution (CaSLiDE) for evolving highly	Duran Sürür
	efficient and specific miniature CRISPR-Cas systems	Medical Faculty a
	12:45 - 13:05	<u>ID 1</u> 2
	Revolutionizing CRISPR/Cas9 Gene Therapy with Targeted Chemical Modulation	Xinlai Cheng Buchmann institut
	13:05 - 13:25	ID 143
	Xdrop®: changing the approach to gene editing validation and single-cell functional	Sidsel Alsing
	assays	Sampli
13:30 - 14:30	Lunch Break	
14:30 - 14:45	Clasing address by CMNI	
	Closing address by CMN	
15:00 - 17:00	Main Stage	
	Closing social event	
	Main Stage Optional Social Event: Guided heat trip in the Cononhagen sanals	
16:00 - 17:00	Optional Social Event: Guided boat trip in the Copenhagen canals	
	Copenhagen	