

TUESDAY 27 OCTOBER

08:30 · Entrance Hall

EDUCATION SESSION / CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP REGISTRATION

09:00-10:45 · PARALLEL · Hall 3, Level 0

EDUCATION SESSION 1a - Expanding the delivery possibilities by the design of new vectors for gene therapy
Chair: *Hildegard Büning, Hannover Medical School*

Welcome

09:05 **Jessica Hartmann, PEI Langen****EDU01:** Targeted lentiviral vectors for in vivo CAR-T cell generation09:30 **Hildegard Büning, Hannover Medical School****EDU02:** Adeno-associated virus (AAV) vectors – Insights into strategies for overcoming limitations in host-vector interactions09:55 **Anja Ehrhardt, University of Witten/Herdecke****EDU03:** The Science of Adenoviral Vectors: How Do They Work and Why Do They Matter?10:20 **Roy van der Meel, Eindhoven University of Technology****EDU04:** LNP technology enabling vaccines, immunotherapies & gene editing

09:00-10:45 · PARALLEL · Hall 4, Level 0

EDUCATION SESSION 1b - Therapeutic strategies expanding the horizons of GT
Chair: *Gloria Gonzalez-Aseguinolaza, CIMA, University of Navarra, Pamplona*

Welcome

09:05 **Hinrich Abken, University of Regensburg****EDU05:** Augmenting CAR T cell capacities by synthetic circuits09:30 **Claudio Mussolino, University of Freiburg****EDU06:** Epigenome editing09:55 **Alessia Cavazza, University College London****EDU07:** VLPs for in vivo CAR-T cells10:20 **Matthis Synofzik, University of Tübingen****EDU08:** Antisense Oligonucleotides: Programmable Medicines Beyond Gene Therapy

10:45-11:15 · Hall H

EDUCATION SESSION COFFEE BREAK

11:15-13:00 · PARALLEL · Hall 3, Level 0

EDUCATION SESSION 2a - What human trials are teaching us
Chair: *Hildegard Büning, Hannover Medical School*11:15 **David Williams, Boston Children's Hospital****EDU09:** Long-term effects of gene therapy11:40 **Gloria Gonzalez Aseguinolaza, CIMA, University of Navarra, Pamplona****EDU10:** Side effects and protein stress response12:05 **Giuseppe Ronzitti, Genethon, Evry****EDU11:** Immune responses and redosing possibilities12:30 **Spela Milosevicz, CTNNB1 foundation, Gen2Cure****EDU12 EKFS Award winner:** Ultrarare diseases and patient access challenges



11:15-13:00 · PARALLEL · Hall 4, Level 0

EDUCATION SESSION 2b - Expanding GT models for mechanisms, prediction and data analysis
Chair: *Anne Galy, ART-TG – Inserm US35*11:15 **Giacomo Domenici, iBET, Oeiras****EDU13:** Human 3D Microenvironment Models to Assess Responses to Gene and Cell Therapies11:40 **Helena Zacharias, Hannover Medical School****EDU14:** Artificial Intelligence: Opportunities and Challenges for Biomedical Research12:05 **Michael Rothe, Hannover Medical School****EDU15:** Safety evaluations of CGT products in vitro and in vivo12:30 **Mauro Giacca, Kings College London****EDU16:** Disease models, epigenetic and age factors of susceptibility to AAV



09:30-11:00 · WORKSHOP · Hall G, Level 2

CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP 1
Chairs: *Alessandro Aiuti, SR-Tiget, Milan; Claire Booth, University College London*

Welcome and introduction

09:40 **Carmen Sanges, Universitätsklinikum Würzburg****CTC01:** From Vein-to-Vein to On-Demand: Rethinking Manufacturing Infrastructure for Advanced Therapies10:00 **Paolo Morgese, Alliance for Regenerative Medicine****CTC02:** Real world evidence generation for MAA and HTA10:20 **Hans Dieter Volk, Charité Universitätsmedizin Berlin and CheckImmune GmbH****CTC03:** ID card of gene and cell therapy products and therapy response monitoring

10:40 Roundtable and Q&A

11:00-11:15 · Hall H

CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP COFFEE BREAK

11:15-13:00 · WORKSHOP · Hall G, Level 2

CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP 2
Chairs: *Alessandro Aiuti, SR-Tiget, Milan; Claire Booth, University College London*11:15 **Anna-Pia Papageorgiou, EU****CTC04:** The ATMP centers of excellence and EU biotech-act11:35 **Stefano Benvenuti, Fondazione Telethon****CTC05:** Not for profit model of ATMP commercialization in EU and US11:55 **Chris Sampson, OHE consulting****CTC06:** The case for system-level investment in cell and gene therapies in the UK12:15 **Johan Van Eldere, European University Hospital Alliance****CTC07:**

12:35 Roundtable and Q&A

TUESDAY 27 OCTOBER	
12:00-14:00 · Entrance Hall	REGISTRATION
14:00-16:30 · PLENARY · Hall 1	SESSION 1: ESGCT 2026 Opening: Clinical applications of gene therapy <i>Chairs: Alberto Auricchio, Tigem, Naples; Zoltan Ivics, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig; Boris Fehse, University of Hamburg</i> Alberto Auricchio, Tigem, Naples; Zoltan Ivics, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig; Boris Fehse, University of Hamburg Welcome 14:30 Dimitrios Mougiakakos, Universitätsklinikum Magdeburg A.ö.R. INV01: 15:00 Giuliana Ferrari, SR-Tiget, Milan INV02: Impact of HSC signature on gene therapy for beta-thalassemia 15:30 William Schief, Scripps Research, San Diego INV03: 16:00 Michel Sadelain, Columbia University INV04:
16:30-17:00 · Hall H	COFFEE BREAK
17:00-19:30 · PARALLEL · Hall 1	SESSION 2a: AAVs/Non integrative vectors I - Basic <i>Chairs: Isabelle Richard, Genethon, Evry; Anna Marei Mann, UKE, Hamburg</i> 17:00 Mario Mietzsch, University of Florida INV05: Structural characterization of the AAV capsid interactome 17:30 Silvana Libertini, Novartis INV06: Long-read TES to study rAAV integration: from benchmarking in clonal systems to insights in primary human hepatocytes 18:00 Pedram Moeini, CIMA, University of Navarra, Pamplona OR001: Molecular mechanism of severe hepatotoxicity following high-dose ssAAV9.CBA.SMN1 administration in neonatal mice 18:15 Adrian Westhaus, Genethon, Evry OR002: Luminal optimization of the VP1/2 N-termini for improved transduction sheds light on AAV5's trafficking bottlenecks 18:30 Dimitri Romanovsky, LMU, Munich OR003: Affinity proteomics-based receptor screening reveals capsid-dependent host interactions of AAV vectors in the retina 18:45 Victoria Xu, Imperial College London OR004: Structural basis for Rep-mediated adeno-associated virus packaging. 19:00 Presented posters P0019, P0112, P0124, P0152
17:00-19:30 · PARALLEL · Hall G, Level 2	SESSION 2b: (Epi)genome editing I <i>Chairs: Frank Buchholz, TUDresden; Guillermo Montoya, University of Copenhagen</i> 17:00 Jacob Giehm Mikkelsen, Aarhus University INV07: 17:30 Angelo Lombardo, SR-Tiget, Milan INV08: 18:00 Yaolin Pu, Charité-Universitätsmedizin Berlin OR005: Adenine base editing of <i>FKBP12</i> and <i>NR3C1</i> creates tacrolimus- and glucocorticoid-resistant T cells and Tregs for transplant immunotherapy 18:15 Jason Fernandes, Scribe Therapeutics OR006: Next generation CasX-based epigenetic silencers engineered for potency, specificity, and durability: from high-throughput discovery to clinical translation 18:30 Alessio Conci, University of Trento OR007: In Vivo Homology-Independent Targeted Integration Mediated by All-in-One AAV Delivery of a Novel Engineered TnpB Genome Editor 18:45 Xu Hui, Refrogene Medicine, OR008: Durable clinical benefits with RM001 (Autologous HBG1/2 Promoter-modified CD34+ Hematopoietic Stem and Progenitor Cells) for transfusion-dependent beta-thalassemia 19:00 Presented posters P0906, P0923, P0992, P1019

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17:00-19:30 · PARALLEL · Hall 3, Level 0

SESSION 2c: Immunotherapy and CAR T cells IChairs: **Bernhard Gentner**, Centre Hospitalier Universitaire Vaudois (CHUV); **Michael Hudecek**, Universität Würzburg**17:00 Haig Aghajanian**, University of Pennsylvania**INV09:****17:30 Evelyn Ulrich**, University Hospital Frankfurt**INV10:** Genetic engineering in NK cells: unleashing the killer instinct for immunotherapy**18:00 Elena Sotillo**, Stanford University**OR009:** Codelivery of IL9 to enhance engraftment and potency of in vivo engineered CAR-T cells generated via CD3-targeting lentivirus.**18:15 Silvan Brunn**, University Hospital Zurich**OR010:** LILRB2 targeting enhances CAR T cell efficacy in acute myeloid leukemia**18:30 Marc Felde**, Hannover Medical School**OR011:** Cell-based therapy for amyloid pathology in Alzheimer's disease using engineered iPSC-Macs**18:45 Niklas Wiese**, BIH, Charité, Berlin**OR012:** A Pan-Viral GMP-compatible CRISPR-Cas9-based platform for Tacrolimus-resistant antiviral T-cell therapies after solid organ transplantation**19:00 Presented posters****P0291, P0316, P0317, P0356**

17:00-19:30 · PARALLEL · Hall 4, Level 0

SESSION 2d: Metabolic diseases I

In collaboration with JSGCT and CSGCT

Chairs: **Nicola Brunetti-Pierri**, Tigem, Naples; **Gloria Gonzalez-Aseguinolaza**, CIMA, University of Navarra, Pamplona**17:00 Hao Yin**, Shanghai Changzheng Hospital**INV11:** Islet and Endodermal Stem cell-derived Islet Transplant in T1D and T2D**17:30 Sabine Fuchs**, University of Utrecht**INV12:** Prime Time for patients with metabolic diseases**18:00 Hiroshi Kobayashi**, The Jikei School of Medicine, Tokyo**INV13:** Research and Development of Gene Therapy for Lysosomal Storage Diseases**18:30 Stefania Crippa**, SR-TIGET, Milan**OR013:** Preclinical development of lentiviral-based hematopoietic stem/progenitor cells (HSPC)-gene therapy (GT) for Mucopolysaccharidosis type IVA within an innovative platform program for Lysosomal Storage Disorders.**18:45 Juliette Lemoine**, Inserm UMR_S1163, Institut Imagine, Paris**OR014:** Muscle-targeted and liver detargeted AAV gene therapy provides sustained correction of MSUD in neonatal mice**19:00 Presented posters****P0203, P0204, P0206, P0209, P0212**

19:30-21:00 · Hall H

WELCOME RECEPTION

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08:00-08:30 · Entrance Hall
REGISTRATION

08:30-10:30 · PARALLEL · Hall 1

SESSION 3a: AAVs/non integrative vectors II - Technology

Chairs: Nerea Zabaleta Lasarte, CIMA, University of Navarra, Pamplona; Paula Sureda Horrach, Tigem Naples

08:30 Anja Ehrhardt, Witten/Herdecke University

INV14: Engineering next-generation adenoviral vectors for improved delivery: new strategies and emerging opportunities

09:00 Moin Saleem, University of Bristol

INV15: Gene therapy for kidney disease, current technology and future perspectives

09:30 Sourav Choudhury, Sanofi

OR015: MADCAP: Unearthing Metagenomic AAV Caches with Deep Petascale Mining

09:45 Songpu Xie, University Medical Center Utrecht

OR016: NEO-AAV: an engineered extracellular vesicle-enveloped AAV platform for activation-coupled T cell transduction and CAR-T cell generation

10:00 Xabier Perez Garmendia, Hannover Medical School

OR017: In vivo targeting of hematopoietic stem cells by capsid-engineered AAV vectors

10:15 Jan Bieri, Heidelberg University

OR018: Erythroid progenitor-specific gene delivery using an adaptable AAV targeting system

08:30-10:30 · PARALLEL · Hall G, Level 2

SESSION 3b: Hematopoietic & PID

Chairs: Giorgia Santilli, University College London; Dan Bauer, Broad Institute

08:30 Alessandro Aiuti, SR-Tiget, Milan

INV16: Wiskott–Aldrich Syndrome: Translating Gene Therapy into Standard of Care

09:00 Annarita Miccio, Institut Imagine, Paris

INV17: Gene Therapy of β -Hemoglobinopathies: Successes and Challenges

09:30 Stefano Gritti, University College London

OR019: Patient-specific gene editing approaches rescue B cell development in X-linked agammaglobulinemia

09:45 Beatriz Olalla-Sastre, Ciemat/Ciberer/IIS Funcacion Jimenez Diaz, Madrid

OR020: Prime editing-driven correction of Fanconi anemia mutations in human HSPCs

10:00 Serena Scala, SR-Tiget, Milan

OR021: Biological properties and clonality of engineered hematopoietic stem/progenitor cells persisting long-term after gene therapy

10:15 Kiriaki Paschoudi, Aristotle University of Thessaloniki

OR022: In vivo HbF reactivation and selective erythroid enrichment using a novel adenoviral platform

08:30-10:30 · PARALLEL · Hall 3, Level 0

SESSION 3c: Oncolytic therapy & cancer gene therapy

Chairs: Anja Ehrhardt, Witten/Herdecke University; Vincenzo Cerullo, University of Helsinki

08:30 Akseli Hemminki, University of Helsinki

INV18: Oncolytic adenoviruses in humans, what have we learned from treatment of > 500 patients with viruses such as Oncos-102 and TILT-123?

09:00 Dirk Nettelbeck, DKFZ Heidelberg

INV19: Oncolytic Virus Development: Personalized Approaches and Inducible Payloads

09:30 Iñaki Beasain, CIMA, University of Navarra, Pamplona

OR023: Preliminary results of a phase I trial evaluating DNX-2440 oncolytic adenovirus in recurrent glioblastoma

10:00 Firas Hamdan, University of Helsinki

OR024: Leveraging trained immunity for the epigenetic and metabolic rewiring of macrophages to enhance colorectal cancer vaccine response

10:30 Ignacio Sallent, University of Barcelona, Bellvitge Biomedical Research Institute (IDIBELL)

OR025: ATF6-Driven enhancement of oncolytic virus efficacy and immunogenicity

11:00 Rebekka Medert, Akribion Therapeutics

OR026: Next-Generation Cancer Targeting: Harnessing CRISPR-Cas12a2 to Eliminate HPV-Positive Tumor Cells.

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08:30-10:30 · PARALLEL · Hall 4, Level 0

SESSION 3d: Non-viral vectors/nanotechnology and RNA therapeutics IChairs: **Helder Santos**, University of Groningen; **Helena Florindo**, University of Lisbon**08:30 Zoltan Ivics**, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig**INV20:** Advances in *Sleeping Beauty* transposon-based therapeutic cell engineering**09:00 Heinrich Haas**, Neovac, Oxford**INV21:** NeoVac's mRNA Nanoparticle Technologies allowing for better and safer mRNA pharmaceuticals**09:30 Roberto Di Cunto**, Tigem, Naples**OR027:** Advanced LAF-XP polyplexes for Non-Viral Delivery of mRNA to Retinal Photoreceptors**09:45 Sophie Thalmayr**, LMU, Munich**OR028:** Hydrophobic Vining and Surface Modification of Lipo-Xenopeptide mRNA Polyplexes in Different Cell Models In Vitro and In Vivo**10:00 Eoghan O'Leary**, Circio AB**OR029:** circVec: A DNA Vector Platform Harnessing Intracellular Circular RNA Biogenesis for Next-Generation T Cell Therapies**10:15 John Shinu**, Serif Biomedics**OR030:** Modified DNA: a new therapeutic modality for scalable, programmable, durable, and redosable genetic medicines

10:30-11:00 · Hall H

COFFEE BREAK

11:00-12:30 · PARALLEL · Hall 1

SESSION 4a: CNS & sensory IChairs: **Robin Ali**, Kings College London; **Ivana Trapani**, Tigem Naples**11:00 Stuart Cobb**, University of Edinburgh**INV22:** Self-regulating gene therapy for Rett syndrome**11:30 Botond Roska**, Institute of Molecular and Clinical Ophthalmology, Basel**INV23:** Precision therapies for visual diseases**12:00 Stylianos Michalakis**, LMU Munich**OR031:** Clinical translation of a dual AAV gene therapy for ABCA4-associated Stargardt disease**12:15 Amrutha Pattamatta**, Arbor Biotechnologies**OR032:** Single-dose AAV gene editors achieves durable, therapeutically relevant target suppression in the CNS across mice and non-human primates

11:00-12:30 · PARALLEL · Hall G, Level 2

SESSION 4b: Infectious diseases / vaccinesChairs: **Carmen Elena Gomez**, CNB-CSIC, Madrid; **Kristie Bloom**, University of the Witwatersrand**11:00 Britta Eiz-Vesper**, Hanover Medical School**INV24:** Tailored T-cell therapy for viral infections in immunocompromised patients**11:30 Caroline Demangel**, Institut Pasteur, Paris**INV25:****12:00 Dylan Kairuz**, University of the Witwatersrand**OR033:** From Shell to Shot: Refining Nutshell Waste to Synthesise Ionisable Lipids for mRNA-LNP Vaccines**12:15 Yannick Pranghe**, University Medical Center Hamburg-Eppendorf (UKE)**OR034:** A Lentiviral Reporter System for HLA-Restricted Viral CD8+ T-Cell Epitope Screening

11:00-12:30 · PARALLEL · Hall 3, Level 0

SESSION 4c: Immunotherapy and CAR T cells IIChairs: **Waseem Qasim**, University College London; **Sonia Guedan**, IDIBAPS, Barcelona**11:00 He Huang**, Zhejiang University School of Medicine**INV26:****11:30 Andreas Mackensen**, University Hospital of Erlangen**INV27:** Can CAR-T cells cure autoimmune disease?**12:00 Juan Roberto Rodriguez-Madoz**, CIMA, University of Navarra, Pamplona**OR035:** An open CAR-T single-cell atlas to enable in-depth characterization and rational engineering of CAR-T products**12:15 Daniela Cesana**, SR-Tiget Milan**OR036:** Liquid Biopsy Profiling Reveals CAR-T Cell Dynamics, Therapeutic Activity, and Toxicity in B-cell Malignancies

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11:00-12:30 · PARALLEL · Hall 4, Level 0

SESSION 4d: Metabolic diseases II

In collaboration with ASGCT and AGCTS

Chairs: **Matthew Porteus**, Stanford University; **Maria Ester Bernardo**, SR-Tiget, Milan**11:00 Samantha Ginn**, Children Medical Research Institute, Sydney**INV28:** Defining Therapeutic Genome Editing Outcomes in the Liver**11:30 Gerald Schwank**, University of Zurich**INV29:** Correction of Metabolic Liver Diseases by In Vivo Base and Prime Editing**12:00 Michael Byrne**, Wave Life Sciences**OR037:** siRNA SpiNA Design Induces Durable Silencing of Inhbe RNA, Weight loss, and Adipose Remodeling in a Mouse Model of Diet Induced Obesity**12:15 Lucia De Stefano**, Tigem, Naples**OR038:** Development of mRNA replacement therapy in clinically relevant mouse models of Glycogen Storage Disease type 1b

12:30-13:30 · Hall H

LUNCH

13:30-14:30 · Hall G, Level 2

LUNCHTIME SYMPOSIUM: AI and machine learning in gene therapyChairs: **Diego di Bernardo**, Tigem Naples; **Julian Grünewald**, TUM**13:30 Clara Schoeder**, University of Leipzig**INV30:** Computational protein design as strategy for rapid CAR generation**14:00 Guillermo Montoya**, University of Copenhagen**INV31:**

13:30-14:30 · Hall B, Level 1

LUNCHTIME SYMPOSIUM: How to Succeed in Science Publishing**Kevin Davies**, Sage Publishing**Thomas Gallagher**, Human Gene Therapy

13:30-14:30 · Hall 3, Level 0

LUNCHTIME SYMPOSIUM: PlasmidFactory - From DNA to Therapy: Minicircle-Enabled Advances in Cell & Gene TherapyChairs: **Hildegard Büning**, Hannover Medical School / Co-host: **Ram Shankar**, CSO, PlasmidFactory GmbH, Bielefeld**Katrin Mestermann**, Group Leader, Fraunhofer IZI, Würzburg

From Bench to Bedside: Development of a Virus-Free Clinical CAR T Manufacturing Protocol

Dimitrios L. Wagner, Baylor College of Medicine and Charité/Berlin Institute of Health

Non-viral and site-specific integration to create advanced TCR- and CAR-T cell therapies



13:30-14:30 · Hall 4, Level 0

LUNCHTIME SYMPOSIUM: Sartorius - Translating AAV Innovation into Therapeutic Reality: Perspectives from a Developer, Technology Innovator, and CDMOChair: **Szymon Holubowicz**, Technical Sales Gene Therapy, Sartorius**Emily Warner, PhD**, Vice President of Technical Operations**Patrick Erbacher, PhD**, Head of R&D Sartorius Polyplus**Claudio Panzarella**, Director of Business Development ReiThera**Michela Gentile, PhD**, Cell Line Development Supervisor ReiThera

13:30-15:30 · Poster Halls, Level 0 and Level 2

POSTER SESSION I

Uneven posters (P0001, P0003, P0005 etc)

15:30-17:30 · PLENARY · Hall 1

SESSION 5: In vivo gene therapyChairs: **Christian Brendel**, Boston Children's Hospital; **Carmen Unzu**, CIMA, Pamplona**15:30 Christian Buchholz**, PEI, Langen**INV32:** Off-the-shelf CAR T cells through in vivo gene delivery**16:00 Hildegard Büning**, Hannover Medical School**INV33:** Fit to serve - adapting biological nanoparticles by capsid engineering**16:30 Sonia Vallabh**, Broad Institute**INV34:** Progress towards a one-time, brainwide PrP silencing therapy for prion disease**17:00 Viviana Gradinaru**, CalTech**INV35:** Blood-Brain Barrier: Friend and Foe

WEDNESDAY 28 OCTOBER	
17:30-18:00 · Hall H	COFFEE BREAK
18:00-19:00 · PLENARY · Hall 1	SESSION 6: Gene editing <i>Chairs: Paula Rio, CIEMAT, Madrid; Thomas Fox, University College London</i> 18:00 Virginijus Šikšnys , Vilnius University, LT INV36: 18:30 David Liu , Harvard University INV37:

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08:00-08:30 · Entrance Hall

REGISTRATION

08:30-10:30 · PARALLEL · Hall 1

SESSION 7a: Immune responses to GTChairs: **Federico Mingozi**, Nava Therapeutics; **Gwladys Gernoux**, Target, University of Nantes**08:30 Anna Kajaste-Rudnitski**, University of Pavia**INV38:** Insight into cell-autonomous immune responses to AAV transduction**09:00 Carmen Unzu**, CIMA Pamplona**INV39:** Leveraging immune recognition for developing safer and more potent AAV-based gene therapies**09:30 Tiziana La Bella**, Genethon**OR039:** Next-generation IgG degrading enzymes with improved specific activity, broader specificity across species and reduced antigenicity in humans**09:45 Erin Howard**, University of Guelph, Canada**OR040:** Anti-drug antibody responses to AAV-expressed monoclonal antibodies: Effects of antibody choice and vector serotype in a sheep model**10:00 Carlos Andrea Mora**, Hemotune AG**OR041:** Extracorporeal depletion of anti-AAV antibodies in non-human primates with the HemoSystem DEplete**10:15 Giovanna Giacca**, SR-Tiget, Milan**OR042:** In vivo lentiviral engineering of the liver reveals and modulates systemic tumor immune escape

08:30-10:30 · PARALLEL · Hall G, Level 2

SESSION 7b: Genome editing IIChairs: **Toni Cathomen**, University of Freiburg; **Anna Cereseto**, University of Trento**08:30 Christophe Bock**, CeMM Research Center for Molecular Medicine (ÖAW), Vienna**INV40:** Programmed cells? Single-cell biology and cell engineering for immunity and cancer**09:00 Julia Skokowa**, Tübingen University**INV41:** Gene editing for severe congenital neutropenia: preclinical efficacy and the safety considerations for clinical translation.**09:30 Simon Krooss**, Hannover Medical School**OR043 EKFS Award winner:** Clinical Proof-of-Concept for In Vivo Base Editing in PI*ZZ Alpha-1 Antitrypsin Deficiency: Results from the YOLT-202 Phase I/II Trial**09:45 Antony Dinu**, Medical Center University of Freiburg**OR044:** Castling, a novel therapeutic concept for rewiring pathological gene-expression networks, enabled by the TRIPLE technology**10:00 Gregory Cost**, Addition Therapeutics**OR045:** Precise, RNA-mediated insertion of transgenes using an R2 retrotransposase yields stable, durable, and therapeutic hepatic protein expression, from mice to NHPs**10:15 Francesca Morosi**, Tigem, Naples**OR046:** Adenine base editing-mediated MECP2 gene correction as a novel gene therapy strategy for Rett syndrome

08:30-10:30 · PARALLEL · Hall 3, Level 0

SESSION 7c: Accessibility of GTChairs: **Claire Booth**, University College London; **Alessandro Aiuti**, SR-Tiget, Milan**08:30 Sumathi Iyengar**, Wiskott Foundation**INV42:** The Power of Patient Advocacy in Access to Gene Therapy: The Waskyra Journey**08:50 Cornelis Boersma**, University of Groningen**INV43:****09:10 Christopher Baum**, BIH**INV44:****09:30 Jenny Taylor**, University of Oxford**INV45:****09:50 Renske ten Ham**, University Medical Center Utrecht**INV46:** Challenges in market access of GCTs**10:10 Round table discussion**

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08:30-10:30 · PARALLEL · Hall 4, Level 0

SESSION 7d: Skin, pulmonary and skeletal diseasesChairs: **Matthias Titeux**, Institut Imagine; **Galina Boskh**, University of Oxford**08:30 Stephen Hart**, UCL London**INV47:****09:00 Veronica Kinsler**, Crick institute, London**INV48:** Tackling mosaic disorders**09:30 Valentina Capo**, SR-Tiget, Milano**OR047:** Lentiviral vector transduction coupled to ex vivo expansion of circulating hematopoietic stem/progenitor cells to treat a bone disease with limited HSC availability**09:45 Alessandra Fabrizi**, University of Modena and Reggio Emilia**OR048:** Allele-specific CRISPR/Cas9 editing of TP63 paves the way toward precision therapy for EEC-related corneal disease**10:00 Stine Behrmann**, University of Hamburg**OR049:** A unique combination of nucleotide modifications enhances sup-tRNA efficacy in restoring CFTR function**10:15 Dario Gajewski** University Medical Center Göttingen**OR050:** Preclinical development of osteopetrosis gene therapy using an iPSC-derived advanced 3D model of human bone

10:30-11:00 · Hall H

COFFEE BREAK

11:00-13:00 · PLENARY · Hall 1

SESSION 8: Presidential Symposium**In Memoriam: Claudio Bordignon**Chairs: **Alberto Auricchio**, Tigem, Naples; **Zoltan Ivics**, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig**11:00 Alberto Auricchio**, Tigem, Naples**INV49:** Presidential address**11:15 KEYNOTE: Chiara Bonini**, San Raffaele Scientific Institute, Milan; **Alessandro Aiuti**, SR-Tiget, Milan**INV50:****11:45 OUTSTANDING ACHIEVEMENT AWARD - Robin Ali**, King's College London**INV51:** Ocular gene and cell therapy: an eye on the past whilst looking to the future**12:15 CAREER PROGRESSION AWARD - Monica Casucci**, San Raffaele Hospital, Milan**OR051:****12:30 CAREER PROGRESSION AWARD - Thomas Fox**, University College London**OR052:** Prime Time for patients with metabolic diseases**12:45 FRESENIUS** (Martin Zörnig) **AND ZELIG ESHAR** (Reno Debets) **AWARD CEREMONY**

13:00-13:20 · Hall 1

ESGCT AGM

13:00-14:00 · Hall H

LUNCH

13:30-14:30 · Hall 3, Level 0

LUNCHTIME SYMPOSIUM: Cytiva - Solid tumors across modalities: Early decisions that shape the path to patientChair: **Emmanuelle Cameau**, Scientific Director Viral Vectors, Cytiva**Niels-Bjarne Woods Ph.D.**, Associate Professor, Lund Stem Cell Centre, Lund University, Sweden**Chantal Lagresle**, Senior Research Scientist, Imagine Institute for Genetic Diseases, Paris France**Michael Krohn**, Co-CEO & Co-Founder, Akribion Therapeutics**Akseli Hemminki**, Professor of Oncology, University of Helsinki and CEO, TILT Biotherapeutics Oy

13:30-14:30 · Hall 4, Level 0

LUNCHTIME SYMPOSIUM: LonzaChair: **Kelly Molder**, Senior Subject Matter Expert, Lonza**Erin Brooks**, Associate Director, Marketing, Lonza

A simplified, serum-free T cell workflow powered by TheraPEAK® Solutions

Angela DiNardo, Portfolio Manager, Cell and Gene Therapy Media, Lonza

Platform-proof performance for AAV production in HEK293 cell lines

13:30-14:30 · Hall B, Level 1

LUNCHTIME SYMPOSIUM: vfa. and DGGZT Meeting

14:00-16:00 · Poster Halls, Level 0 and Level 2

POSTER SESSION II

Even posters (P0002, P0004, P0006 etc)



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16:00-18:00 · PARALLEL · Hall 1

SESSION 9a: CNS & sensory IIChairs: **Ellen Reisinger**, University of Tübingen; **Assumpció Bosch**, UAB, Barcelona**16:00 Jennifer Marx**, Hannover Medical School**INV52:** Machine learning-guided engineering of AAV2 vectors for selective outer hair cell targeting in the inner ear**16:30 Tobias Moser**, University Medical Center Göttingen**INV53:** Toward optogenetic hearing restoration**17:00 Marija Tomovic**, Tigem, Naples**OR053:** Evaluation of a new mutation-agnostic gene-editing approach for the rhodopsin-associated autosomal dominant Retinitis Pigmentosa in a humanized mouse model**17:15 Deirdre O'Connor**, AviadoBio**OR054:** AVB-406: An intravenous AAV-miRNA achieving sustained MAPT knockdown for the treatment of Alzheimer's disease and other tauopathies**17:30 Atsushi Hoshino**, Kyoto Prefectural University of Medicine**OR055:** A high-potency protective APP variant enables preventive genome editing for Alzheimer's disease**17:45 Tawfeeq Shekh-Ahmad**, The Hebrew University of Jerusalem**OR056:** Activity-regulated and neuron-targeted AAV9-Nrf2 gene therapy suppresses epileptogenesis and chronic epilepsy

16:00-18:00 · PARALLEL · Hall G, Level 2

SESSION 9b: Lentiviral vectors/integrative vectorsChairs: **Pietro Genovese**, Boston Children's Hospital; **Els Verhoeven**, University of Nice**16:00 Melita Irving**, University of Lausanne**INV54:** Designing and Integrating Next-Generation T Cell Strategies With Modular All-in-One Lentiviral Vectors**16:30 Naomi Taylor**, NIH**INV55:****17:00 Marta Pérez Rodríguez**, CIEMAT/CIBERER/IIS Fundación Jiménez Díaz, Madrid**OR057:** In Vivo Lentiviral Gene Therapy for Fanconi Anemia: Systemic Therapeutic Delivery Targeting Key Disease-Relevant Tissues**17:15 Nathan Jeffreys**, Royal Manchester Children's Hospital,**OR058:** Ex-vivo modification of autologous CD34+ HSPCs using a CD11b-directed lentiviral vector encoding ApoEII-tagged human IDS leads to supraphysiological enzyme activity and biochemical correction of neuronopathic MPSII patients**17:30 Danilo Pellin**, Boston Children's Hospital/Dana-Farber Cancer Institute/Harvard Medical School**OR059:** Mapping insertional perturbations to in vivo clonal fitness across human hematopoietic contexts**17:45 Sian Baker**, University of Bristol**OR060:** Autologous haematopoietic stem cell gene therapy for people with Friedreich's Ataxia

16:00-18:00 · PARALLEL · Hall 3, Level 0

SESSION 9c: Immunotherapy and CAR T cells IIIChairs: **Chiara Magnani**, University of Zurich; **Dimitrios Wagner**, Baylor College of Medicine, Houston; **Reno Debets**, ErasmusMC, Rotterdam**16:00 Dirk Busch**, TUM**INV56:** Engineering T cell receptors for clinical applications**16:30 Sine Reker Hadrup**, Technical University of Denmark, Kgs. Lyngby**INV57:****17:00 Niklas Nuppenau**, UMC Hamburg-Eppendorf**OR061:** Discovery and initial characterization of a missense mutation (G1312A; E438K) in a signalling motif of the axi-cel and brexu-cel CD19-CARs present at high frequency in probably all clinical products**17:15 Philip Bucher**, Universitätsklinikum Tübingen - iFIT Cluster of Excellence**OR062 Eshar Adoptive T-cell Therapy Award winner:** ROADSTAR enables CAR design-specific signaling optimization for improved CAR T cell function**17:30 Naiara Martinez Velez**, Stanford University**OR063 Eshar Adoptive T-cell Therapy Award winner:** Tumor-Immune Organoids and Immunocompetent Models Identify Microglial Reprogramming as a Novel Mechanism of cJUN-Enhanced CAR-T Function**17:45 Nina Barceló**, IDIBAPS, Barcelona**OR064:** A versatile microRNA-based platform for safer and precise activation-dependent control of armored CAR-T cell payloads

THURSDAY 29 OCTOBER	
16:00-18:00 · PARALLEL · Hall 4, Level 0	
SESSION 9d: Manufacturing	
<i>Chairs: Ulrike Köhl, Fraunhofer Institute for Cell Therapy and Immunology IZI Leipzig; Matthias Hebben, Complement Therapeutics</i>	
16:00 Michael Hudecek, University Hospital Würzburg	
INV58: New Targets and Technologies for CAR-T Cells	
16:30 Ana Coroadinha, iBET, Oeiras	
INV59: Unlocking AAV Potential: Precision Capsid Engineering for Scalable Manufacturing	
17:00 Gustav Emilsson, AstraZeneca	
INV60: Development and CMC of Lipid Nanoparticles for Non-Viral mRNA Delivery in Gene Editing	
17:30 Raymond Lochner, ULB, Brussels	
OR065: Scale-dependent splicing dynamics and novel isoform discovery during rAAV production	
17:45 Rodrigo Nogueira, iBET, Lisbon	
OR066: Discovery-driven engineering of lentiviral vectors through directed evolution	
18:00-18:30 · Hall H	
COFFEE BREAK	
18:30-19:30 · PLENARY · Hall 1	
SESSION 10: Enabling technologies	
<i>Chairs: Axel Schambach, Hannover Medical School; Juan Roberto Rodriguez-Madoz, Cima Pamplona</i>	
18:30 David Williams, Boston Children's Hospital	
INV61: Leveraging viral vectors from bench to clinic	
19:00 Steve Horvath, Altos Labs	
INV62: Epigenetic Clocks as Biomarkers for Evaluating Cell and Gene Therapy Intervention	
20:00 · Altonaer Fischauktionshalle	
MOLECULAR MINGLE	
	

FRIDAY 30 OCTOBER	
08:30-09:00 · Entrance Hall	REGISTRATION
09:00-10:30 · PARALLEL · Hall 1	SESSION 11a: Cardio & muscular gene therapy <i>Chairs: Seppo Ylä-Herttuala, University of Eastern Finland, Kuopio; Christian Kupatt, TUM Munich</i> 09:00 Markus Rüegg INV63: Mutation-independent dual AAV gene therapy restores near-normal function in LAMA2-related muscular dystrophy 09:30 Roger Hajjar, Mass General Brigham INV64: Breakthroughs in Cardiac Gene Therapy 10:00 Barry Byrne, Powell Gene Therapy Center OR067: Early Results From an Interventional Phase 1 Pediatric Study of ATA-200 Gene Therapy in LGMD-R5 Demonstrate Favorable Safety and Pharmacodynamic Activity at One Year 10:15 Terence Flotte, UMass Chan Medical School OR068: Gene delivery efficiency and toxicity of high systemic dose AAV in a large animal model of Duchenne muscular dystrophy with advanced disease
09:00-10:30 · PARALLEL · Hall G, Level 2	SESSION 11b: Bleeding disorders <i>Chairs: Amit Nathwani, University College London; Alessio Cantore, San Raffaele Telethon Institute for Gene Therapy, Milan</i> 09:00 Wolfgang Miesbach, University of Frankfurt INV65: Current status of gene therapy for haemophilia 09:10 Amit Nathwani, UCL, London INV66: Gene therapy in hemophilia 09:20 Moderated discussion 09:45 Meagan O'Brien, Regeneron INV67: Targeted Gene Insertion of Factor 9 as a Potential Durable Treatment for Hemophilia B 10:15 Antony Floriya, University of Piemonte Orientale OR069: Hepatic endothelium as a safe and durable target for lentiviral gene therapy of hemophilia
09:00-10:30 · PARALLEL · Hall 3, Level 0	SESSION 11c: Disease models <i>Chairs: Mario Amendola, Genethon, Evry; María Tristán Manzano, Genyo Granada</i> 09:00 Monika Gladka, Amsterdam University Medical Center INV69: Translational Perspective for Cardiac Disease Modeling and Therapy Development. 09:30 Andrea Ditadi, SR Tiget, Milan INV70: Charting human developmental hematopoiesis in vitro to open new routes for disease modeling 10:00 Alessia Di Donfrancesco, Fondazione IRCCS Istituto Neurologico Carlo Besta OR070: Ultrasound-Guided Transabdominal Delivery Of AAV9-hSURF1 For Prenatal Treatment Of Leigh Syndrome In Pigs 10:15 Doga Bilen, Regenerative Medicine Center Utrecht, UMC OR071: Back to the Bases: Scalable Modeling of Primary Ciliary Dyskinesia Variants with Base Editing
09:00-10:30 · PARALLEL · Hall 4, Level 0	SESSION 11d: Building the Horizon: European funding for the Cell and Gene Therapies of today and tomorrow <i>Chairs: Janka Matrai, ERC; David Gancberg, HADEA</i> Anna-Pia Papageorgiou, RTD INV71: Horizon Europe for Advanced Therapies: Current EU Landscape and Future Directions Janka Matrai, ERC INV72: The European Research Council's funding opportunities: current developments and future outlook Saverio Tedesco, Crick Institute, London INV73: Advanced in vitro modelling of neuromuscular diseases and gene therapies David Gancberg, HADEA INV74: European Union funding opportunities for Advanced Therapies in Horizon Europe, Cluster 1 Health Annarita Miccio, Institut Imagine, Paris INV75: Assessing efficacy and safety of genome EDITing approaches for Sickle Cell Disease Orsolya Symmons, EIC INV76: Strengthening the Cell and Gene Therapy Pipeline with European Innovation Council support Fabio Rosa, Asgard Therapeutics INV77: Direct cell fate reprogramming for next-generation in vivo immune cell therapies

FRIDAY 30 OCTOBER	
10:30-11:00 · Hall H	COFFEE BREAK
11:00-13:00 · PARALLEL · Hall 1	SESSION 12a: AAVs/non integrative vectors III - Translation <i>Chairs: Luk Vandenbergh, The Broad Institute of Harvard and MIT; Rik Gijssbers, KUL Leuven</i> 11:00 Anne Rosser, University of Cardiff INV78: Using gene therapies in Huntington's disease 11:30 Michelle Rengarajan, Harvard University INV79: 12:00 Sumitava Dastidar, UCL, London OR072: A human multi-organoid framework to improve preclinical assessment of AAV gene therapy in neuromuscular disease 12:15 Rui Nobre, GeneT, University of Coimbra OR073: Cross-species validation of allele-specific silencing for Spinocerebellar Ataxia type 3 12:30 Salvador Rico, Encoded therapeutics OR074: Safety and Efficacy of ETX101, an Investigational AAV9-based Gene Therapy for SCN1A+ Dravet Syndrome: Interim Results from the POLARIS Phase 1/2 Clinical Trials 12:45 Arnaud Valent, Genethon, Evry OR075: Encouraging hepatic safety profile of Genethon's AAV8 microdystrophin gene therapy (GNT0004): cumulative clinical experience from an ongoing clinical trial in ambulant DMD boys
11:00-13:00 · PARALLEL · Hall G, Level 2	SESSION 12b: Genome Editing III <i>Chairs: Juan Bueren, CIEMAT, Madrid; Gerald Schwank, University of Zurich</i> 11:00 Daniel Bauer, Harvard Medical School INV80: Prime assembly targeted genomic integration for blood disorders 11:30 Alessia Cavazza, University of Modena and Reggio Emilia INV81: In Vivo Base Editing for the Treatment of Multisystemic Disorders. 12:00 Jia Chen, ShanghaiTech University/Shanghai Clinical Research and Trial Center OR076: Transformer base-edited autologous CD34+ cells reactivate fetal hemoglobin in β-thalassemia and sickle cell disease 12:15 Romina Marone, University Hospital Basel OR077: Toxin-free replacement of beta-thalassemia hematopoietic stem cells partially reverses disease phenotype in vivo 12:30 Isabell Kassing, Charite, Berlin OR078: One-pot PASTA: A CRISPR-integrase platform for efficient, site-specific integration of large transgenes in primary human T cells 12:45 Deborah Cipria, SR-Tiget, Milano OR079: Simultaneous orthogonal cell engineering by a single CRISPR-Cas9 polyfunctional editor
11:00-13:00 · PARALLEL · Hall 3, Level 0	SESSION 12c: Regulatory & Ethics <i>Chairs: Ilona Reischl, AGES, BASG, Austria; Klaus Cichutek, Goethe University Frankfurt/Main</i> 11:00 Klaus Cichutek, (former President, Paul-Ehrlich-Institut, Langen), Goethe University Frankfurt/Main INV82: AI in ATMP regulations 11:15 Jan Mueller-Berghaus, Paul-Ehrlich-Institut, Langen INV83: Elements and priorities for international regulatory harmonization for ATMPs 11:40 Ilona Reischl-Kok, CAT chair, EMA, AGES Austria INV84: Individualized therapies in the EU 12:05 Michela Gabaldo, VP, Head of ATMP Global Regulatory Affairs, Evotec INV85: ATMP development in a changing Europe: An industry perspective on the EU Pharmaceutical Reform 12:30 Catherine Drai, EMA INV86: Academic ATMP development to marketing authorization: Lenti-WAS (pCCL-CT-WAS) in Wiskott-Aldrich syndrome Panel Discussion

FRIDAY 30 OCTOBER	
11:00-13:00 · PARALLEL · Hall 4, Level 0	SESSION 12d: Non-viral vectors/Nanotechnology and RNA therapeutics II <i>Chairs: Helder Santos, University of Groningen; Zoltan Ivics, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig</i> 11:00 Frank Buchholz, TU Dresden INV87: SLITHER: Evolving genome editing tools where they need to work 11:30 Helena Florindo, University of Lisbon INV88: Modulating Cancer Immunity with RNA-based Nano-Immunotherapies 12:00 Samuel Theuerkauf, Paul-Ehrlich-Institut, Langen OR080: ApoE-DARPin fusion proteins enable selective RNA transfer to target cells by lipid nanoparticles 12:15 Saif Malik, UCL London OR081: Targeted lipid nanocomplex delivery of angiopoietin-1 mRNA slows progression of childhood kidney disease 12:30 Matthias Bozza, CPTx OR082: Targeted immune-silent ssDNA-tLNP delivery enables in vivo CAR-T generation and eradication of disseminated tumors 12:45 Laura Salaberry Abreu, Cima University of Navarra, Pamplona OR083: Comparative study of viral and non-viral delivery systems for cytokine-expressing self-amplifying RNA in cancer therapy
13:00-14:30 · Hall H	LUNCH
14:30-16:30 · PLENARY · Hall 1	SESSION 13 Cancer models & gene therapy <i>Chairs: Luigi Naldini, SR-Tiget, Milan; Monica Casucci, San Raffaele Hospital</i> 14:30 Richard Vile, Mayo Clinic, Rochester INV89: Training T Cells To Traffick To Tumours 15:00 Chiara Bonini, San Raffaele Hospital INV90: 15:30 John Dick, University of Toronto INV91: What makes a stem cell a stem cell and how does it go bad 16:00 Hinrich Abken, Leibniz Institute for Immunotherapy, Regensburg INV92: CARs need the "3rd signal": cytokine enhanced CAR T cells
16:30-16:45 · Hall 1	Concluding remarks
16:45-17:30 · Entrance Hall	Farewell drinks