## 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 generation

**09:30 Hildegard Büning,** *Hannover Medical School*
**EDU02:** Adeno-associated virus (AAV) vectors – Insights into strategies for overcoming limitations in host-vector interactions

**09:55 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 circuits

**09:30 Claudio Mussolino,** *University of Freiburg*
**EDU06:** Epigenome editing

**09:55 Alessia Cavazza**, *University College London*
**EDU07:** VLPs for in vivo CAR-T cells

**10:20 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

VIRALGEN

*Chair: **Hildegard Büning**, Hannover Medical School*

**11:15 David Williams,** *Boston Children's Hospital*
**EDU09:** Long-term effects of gene therapy

**11:40 Gloria Gonzalez-Aseguinolaza**, *CIMA, University of Navarra, Pamplona*
**EDU10:** Side effects and protein stress response

**12:05 Giuseppe Ronzitti,** *Genethon, Evry*
**EDU11:** Immune responses and redosing possibilities

**12:30 Špela Miroševič,** *CTNNB1 foundation, Gene2Cure*
**EDU12 Fresenius Foundation 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 Therapies

**11:40 Helena Zacharias**, *Hannover Medical School*
**EDU14:** Artificial Intelligence: Opportunities and Challenges for Biomedical Research

**12:05 Michael Rothe,** *Hannover Medical School*
**EDU15:** Safety evaluations of CGT products in vitro and in vivo

**12:30 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 Therapies

**10:00 Paolo Morgese,** *Alliance for Regenerative Medicine*
**CTC02:** Real world evidence generation for MAA and HTA

**10:20 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:** EU policies supporting ATMP clinical development and commercialization

**11:35 Stefano Benvenuti**, *Fondazione Telethon*
**CTC05:** Not for profit model of ATMP commercialization in EU and US

**11:55 Chris Sampson,** *OHE consulting*
**CTC06:** The case for system-level investment in cell and gene therapies in the UK

**12:15 Johan Van Eldere**, *European University Hospital Alliance*
**CTC07**: An academic pathway for development of ATMPs

**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**

*Chairs:* ***Alberto Auricchio****, Tigem, Naples;* ***Zoltan Ivics,*** *Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig;* ***Boris Fehse,*** *University of Hamburg*

**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:** T cell-mediated B cell depletion in autoimmunity

**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:** Germline-targeting vaccine design to elicit HIV broadly neutralizing antibodies

**16:00 Michel Sadelain,** *Columbia Institute for Cell Engineering and Therapy (CICET), New York, NY*
**INV04**: Origins and Evolution of Chimeric Antigen Receptors

16:30-17:00 · Hall H
**COFFEE BREAK**

17:00-19:30 · PARALLEL · Hall 1
**SESSION 2a: AAVs/Non integrative vectors I - Basic**

*Chairs:* ***Isabelle Richard****, Genethon, Evry;* ***Anna Marei Mann****, UKE, Hamburg*

**17:00 Mario Mietzsch,** *University of Florida*
**INV05:** Structural characterization of the AAV capsid interactome

**17:30 Silvana Libertini,** *Novartis*
**INV06:** Investigating DNA damage response and viral integration in rAAV-transduced 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**

*Chairs:* ***Frank Buchholz,*** *TUDresden;* ***Guillermo Montoya,*** *University of Copenhagen*

**17:00 Jacob Giehm Mikkelsen,** *Aarhus University*
**INV07:** Prime editing in hematopoietic stem cells for correction of GATA2 deficiency – from all-RNA nucleofection to RNP delivery using lentivirus-derived VLPs

**17:30 Angelo Lombardo,** *SR-Tiget, Milan*
**INV08:** Programming Durable Gene Silencing by Targeted Epigenetic Editing.

**18:00 Yaolin Pu,** *Charité-Universitätsmedizin Berlin*
**OR005:** Adenine base editing of *FKBP12* and *NR3C1* 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 Hui Xu,** *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**

## TUESDAY 27 OCTOBER

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

### SESSION 2c: Immunotherapy and CAR T cells I

*Chairs:* ***Bernhard Gentner,*** *Centre Hospitalier Universitaire Vaudois (CHUV);* ***Katrin Mestermann,*** *Fraunhofer Institute for Cell Therapy and Immunology IZI, Würzburg*

**17:00 Dimitrios Wagner,** *Baylor College of Medicine & Charité / Berlin Institute of Health*
**INV09:** Engineering CAR T cells by targeted integration – and taking it in vivo

**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

## WEDNESDAY 28 OCTOBER

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; **Daniel 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 Fundación Jiménez Díaz, 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: **Martine Lamfers**, Erasmus MC, Rotterdam; **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

**09:45 Firas Hamdan,** *University of Helsinki*
**OR024:** Leveraging trained immunity for the epigenetic and metabolic rewiring of macrophages to enhance colorectal cancer vaccine response

**10:00 Ignacio Sallent,** *University of Barcelona, Bellvitge Biomedical Research Institute (IDIBELL)*
**OR025:** ATF6-Driven enhancement of oncolytic virus efficacy and immunogenicity

**10:15 Rebekka Medert,** *Akribion Therapeutics*
**OR026:** Next-Generation Cancer Targeting: Harnessing CRISPR-Cas12a2 to Eliminate HPV-Positive Tumor Cells.

## WEDNESDAY 28 OCTOBER

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

### SESSION 3d: Non-viral vectors/nanotechnology and RNA therapeutics I

cytiva

*Chairs:* ***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 Tuning and Surface Modification of Lipo-Xenopeptide mRNA Polyplexes in Different Cell Models In Vitro and In Vivo

**10:00 Thomas Hansen,** *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 I

*Chairs:* ***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 / vaccines

*Chairs:* ***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:** An immunopeptidomics-based mRNA vaccine for Tuberculosis

**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 II

*Chairs:* ***Waseem Qasim,*** *University College London;* ***Elena Sotillo,*** *Stanford University*

**11:00 He Huang,** *Zhejiang University School of Medicine*
**INV26:** Functional Optimization, Transplant Integration, and Indication Expansion of CAR-T Cell Therapy

**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

## WEDNESDAY 28 OCTOBER

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 Gloria Gonzalez-Aseguinolaza,** *CIMA, University of Navarra, Pamplona*
**INV29:** Correction of Metabolic Liver Diseases by AAV liver Gene Editing and its molecular implications

**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 therapy

*Chairs:* ***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:** Decoding the molecular mechanisms of CRISPR-associated transposons to reset genome engineering

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 Therapy

*Chairs:* ***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 CDMO

*Chair:* ***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 therapy

*Chairs:* ***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**

*Chairs:* ***Paula Rio****, CIEMAT, Madrid;* ***Thomas Fox****, University College London*

**18:00 Virginijus Šikšnys,** *Vilnius University, LT*
**INV36:** Enhanced compact nucleases as next generation therapeutic genome editors

**18:30 David Liu,** *Harvard University*
**INV37:** Mutation-Specific, Mutation-Agnostic, and Disease-Agnostic Therapeutic Genome Editing

## THURSDAY 29 OCTOBER

08:00-08:30 · Entrance Hall
**REGISTRATION**

08:30-10:30 · PARALLEL · Hall 1
**SESSION 7a: Immune responses to GT**

*Chairs:* ***Federico Mingozzi,*** *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 II**

*Chairs:* ***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** ***Fresenius Foundation 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 GT**

*Chairs:* ***Claire Booth****, University College London;* ***Alessandro Aiuti****, SR-Tiget, Milan*

**08:30 Sumathi Iyengar,** *Wiskott Foundation*
**INV42:** Bringing the Patient Voice into Gene Therapy: Lessons from Wiskott–Aldrich Syndrome

**08:50 Cornelis Boersma,** *University of Groningen*
**INV43:** Healthcare systems fit for cell and gene therapies

**09:10 Christopher Baum,** *BIH*
**INV44:** Cross-institutional and Cross-border Synergies to Enhance Access to Gene Therapy

**09:30 Jenny Taylor,** *University of Oxford*
**INV45:** Genomic Diagnostics to Therapeutics; the UK's Rare Therapies Launch Pad

**09:50 Chris Sampson**, *OHE consulting*
**INV46**: The case for system-level investment in cell and gene therapies in the UK

**10:10 Round table discussion**

## THURSDAY 29 OCTOBER

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

**SESSION 7d: Skin, pulmonary and skeletal diseases**

*Chairs:* ***Matthias Titeux****, Institut Imagine;* ***Galina Boskh****, University of Oxford*

**08:30 Stephen Hart,** *UCL London*
**INV47:** RNA and Gene Editing Therapies for Genetic Respiratory Disease: Overcoming the Delivery Challenge

**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

**In Memoriam: Claudio Bordignon**

**SESSION 8: Presidential Symposium**

*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:** Claudio Bordignon: in Memoriam

AGC Biologics
Your friendly CDMO expert

**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:** Unlocking the full potential of CAR-T cell therapy

**12:30 CAREER PROGRESSION AWARD - Thomas Fox,** *University College London*
**OR052:** Engineering immunity: from gene correction to programmable cell therapy

**12:45 FRESENIUS FOUNDATION** (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 patient**

cytiva

*Chair:* ***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*
**Mario Squadrito,** *Group Leader, SR-Tiget*

13:30-14:30 · Hall 4, Level 0

**LUNCHTIME SYMPOSIUM: Lonza - Two Frontiers. One Future. Advancing Cell and Gene Therapy from Discovery to Impact.**

Lonza

*Chair:* ***Erin Brooks****, Associate Director, Marketing, Lonza*

**Gabriel Gata,** *Associate Director, Subject Matter Expert, Cell and Gene Therapy Media, 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 - How can we create synergies to advance gene therapies?**

*Chair:* ***Tim Flink****, vfa (Verband Forschender Arzneimittelhersteller; German Association of Research-Based Pharmaceutical Companies)*

**Andrea Lübcke,** *MdB (Member of the German Parliament), Germany*
**Hildegard Büning,** *Scientific Secretary, German Society of Gene and Cell Therapy (DGGZT), Institute of Experimental Hematology, Hannover Medical School, Germany*
**Christopher Baum,** *Spokesperson, German National Strategy for Gene- and Cell-based Therapies, Berlin Institute of Health, Berlin, Germany*
**Ulrike Köhl,** *Member of the Board of DGGZT, Fraunhofer IZI, Leipzig, Germany*
**Christian Kupatt,** *Vice President of DGGZT, Klinikum rechts der Isar, TU Munich, Germany*

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

**POSTER SESSION II**

*Even posters (P0002, P0004, P0006 etc)*

AskBio
A Bayer Cell & Gene Therapy Platform Company

## THURSDAY 29 OCTOBER

16:00-18:00 · PARALLEL · Hall 1

### SESSION 9a: CNS & sensory II

*Chairs:* ***Ellen Reisinger,*** *University of Tübingen;* ***Assumpció Bosch****, UAB, Barcelona*

**16:00 Jennifer Marx**, *Hannover Medical School*
**INV52:** Design, Learn, Evolve: AAV Engineering for the Inner Ear

**16:30 Tobias Moser,** *Else Kröner Fresenius Center for Optogenetic Therapies*
**INV53:** Optogenetic therapies: opportunities and challenges

**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 vectors



*Chairs:* ***Pietro Genovese****, Boston Children's Hospital,* ***Els Verhoeyen,*** *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:** Fueling CAR T Cells to Overcome Metabolic Stress

**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 III



*Chairs:* ***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:** Novel tools to modulate T cell recognition and function

**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**

*Chairs:* ***Ulrike Köhl,*** *Fraunhofer Institute for Cell Therapy and Immunology IZI Leipzig;* ***Matthias Hebben****, Complement Therapeutics*

**16:00 Michael Hudecek,** *University Hospital Würzburg*
**INV58:** New developments for CAR-T cell therapies

**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**

*Chairs:* ***Axel Schambach****, Hannover Medical School;* ***Juan Roberto Rodriguez-Madoz,*** *Cima Pamplona*

**18:30 David Williams,** *Boston Children's Hospital*
**INV61:** Leveraging viral vectors from bench to clinic

**19:00 Steve Horvath,** *Department of Medicine, Division of Geriatrics, David Geffen School of Medicine, University of California, Los Angeles, USA*
**INV62:** Epigenetic Clocks as Biomarkers for Evaluating Cell and Gene Therapy Interventions

20:00 · Altonaer Fischauktionshalle

**MOLECULAR MINGLE**

cytiva

## FRIDAY 30 OCTOBER

08:30-09:00 · Entrance Hall
**REGISTRATION**

09:00-10:30 · PARALLEL · Hall 1
**SESSION 11a: Cardio & muscular gene therapy**

*Chairs:* ***Seppo Ylä-Herttuala,*** *University of Eastern Finland, Kuopio;* ***Christian Kupatt****, TUM Munich*

**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**

*Chairs:* ***Amit Nathwani,*** *University College London;* ***Alessio Cantore,*** *San Raffaele Telethon Institute for Gene Therapy, Milan*

**09:00 Wolfgang Miesbach,** *University of Frankfurt*
**INV65:** Current status of gene therapy in hemophilia – the German experience

**09:10 Amit Nathwani,** *UCL, London*
**INV66:** Current status of gene therapy in hemophilia – the UK experience

**09:20 Moderated discussion:** Uptake of hemophilia gene therapy in the real world

**09:45 Meagan O'Brien,** *Regeneron*
**INV67:** Targeted gene insertion of *Factor 9* as a potential durable treatment for haemophilia 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**

*Chairs:* ***Mario Amendola****, Genethon, Evry;* ***María Tristán Manzano****, Genyo Granada*

**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**

*Chairs:* ***Janka Mátrai,*** *ERC;* ***David Gancberg****, HADEA*

**Anna-Pia Papageorgiou,** *RTD*
**INV71:** Horizon Europe for Advanced Therapies: Current EU Landscape and Future Directions

**Janka Mátrai,** *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**

*Chairs: **Luk Vandenberghe,** The Broad Institute of Harvard and MIT; **Rik Gijsbers**, KUL Leuven*

**11:00 Anne Rosser,** *University of Cardiff*
**INV78:** Using gene therapies in Huntington's disease

**11:30 Michelle Rengarajan,** Massachusetts General Hospital
**INV79:** Addressing AAV Seropositivity Through an Interdisciplinary Industry-Academic Consortium

**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**

*Chairs: **Juan Bueren**, CIEMAT, Madrid; **Francisco Martin**, Genyo, Granada*

**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**

*Chairs: **Ilona Reischl**, AGES, BASG, Austria; **Klaus Cichutek,** Goethe University Frankfurt/Main*

**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**

*Chairs: **Helder Santos**, University of Groningen; **Zoltan Ivics**, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig*

**11:00 Frank Buchholz,** *TU Dresden*
**INV87:** Gene editing platforms for innovative health solutions

**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**

*Chairs: **Luigi Naldini**, SR-Tiget, Milan; **Axel Schambach**, Hannover Medical School*

**14:30 Richard Vile,** *Mayo Clinic, Rochester*
**INV89:** Training T Cells To Traffick To Tumours

**15:00 Chiara Bonini,** *San Raffaele Hospital*
**INV90:** Harnessing T-cell exhaustion by genome editing

**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**