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: The ATMP centers of excellence and EU biotech-act

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: 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

*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); **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

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

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 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 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;* ***Sonia Guedan,*** *IDIBAPS, Barcelona*

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 Renske ten Ham, *University Medical Center Utrecht*
INV46: Challenges in market access of GCTs

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:

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

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, *Altos Labs*
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; **Monica Casucci**, San Raffaele Hospital*

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