

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 Matthias 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 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 Mirošević, 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: 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 <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 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 IChairs: **Bernhard Gentner**, Centre Hospitalier Universitaire Vaudois (CHUV); **Michael Hudecek**, Universität Würzburg**17:00 Dimitrios Wagner**, 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 JSGT 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 - TechnologyChairs: **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 & PIDChairs: **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 therapyChairs: **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 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 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 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:** 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 IIChairs: **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 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:** 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 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: 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 <i>Chairs: Federico Mingozi, Nava Therapeutics; Gwladys Gernoux, Target, University of Nantes</i> 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 <i>Chairs: Toni Cathomen, University of Freiburg; Anna Cereseto, University of Trento</i> 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: Casting, 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 <i>Chairs: Claire Booth, University College London; Alessandro Aiuti, SR-Tiget, Milan</i> 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 <i>Chairs: Matthias Titeux, Institut Imagine; Galina Boskh, University of Oxford</i> 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 SESSION 8: Presidential Symposium <i>Chairs: Alberto Auricchio, Tigem, Naples; Zoltan Ivics, Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig</i> 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 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	In Memoriam: Claudio Bordignon 
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 <i>Chair: Emmanuelle Cameau, Scientific Director Viral Vectors, Cytiva</i> 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. <i>Chair: Erin Brooks, Associate Director, Marketing, Lonza</i> 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? <i>Chair: Tim Flink, vfa (Verband Forschender Arzneimittelhersteller; German Association of Research-Based Pharmaceutical Companies)</i> 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 <i>Even posters (P0002, P0004, P0006 etc)</i>	

THURSDAY 29 OCTOBER

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:** 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 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:** Fueling CAR T Cells to Overcome Metabolic Stress**17:00 Marta Pérez Rodríguez, CIEMAT/CIBERER/IIIS 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 Friedrich'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:** 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 <i>Chairs: Ulrike Köhl, Fraunhofer Institute for Cell Therapy and Immunology IZI Leipzig; Matthias Hebben, Complement Therapeutics</i> 16:00 Michael Hudecek, <i>University Hospital Würzburg</i> INV58: New developments for CAR-T cell therapies 16:30 Ana Coroadinha, <i>iBET, Oeiras</i> INV59: Unlocking AAV Potential: Precision Capsid Engineering for Scalable Manufacturing 17:00 Gustav Emilsson, <i>AstraZeneca</i> INV60: Development and CMC of Lipid Nanoparticles for Non-Viral mRNA Delivery in Gene Editing 17:30 Raymond Lochner, <i>ULB, Brussels</i> OR065: Scale-dependent splicing dynamics and novel isoform discovery during rAAV production 17:45 Rodrigo Nogueira, <i>iBET, Lisbon</i> 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, <i>Boston Children's Hospital</i> INV61: Leveraging viral vectors from bench to clinic 19:00 Steve Horvath, <i>Department of Medicine, Division of Geriatrics, David Geffen School of Medicine, University of California, Los Angeles, USA</i> INV62: Epigenetic Clocks as Biomarkers for Evaluating Cell and Gene Therapy Interventions	
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 therapyChairs: **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 disordersChairs: **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 modelsChairs: **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 tomorrowChairs: **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 Vandenbergh, The Broad Institute of Harvard and MIT; Rik Gijssbers, 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 <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: 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 <i>Chairs: Luigi Naldini, SR-Tiget, Milan; Axel Schambach, Hannover Medical School</i> 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