TUESDAY 7 OCTOBER

08:30 · Registration Foyer
EDUCATION SESSION / CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP REGISTRATION

09:00-10:45 · PARALLEL · Room Parallel A
EDUCATION SESSION 1a - Gene delivery: going viral and non-viral
Chairs: ***Hildegard Büning,*** *Hannover Medical School*

09:00 Welcome

09:05 Hildegard Büning, *Hannover Medica School*
EDU01*:* Insights on the adeno-associated virus (AAV) vector system

09:30 Anne Galy, *ART-TG – Inserm US35*
EDU02: Insights on the lentivirus vector system

09:55 Juan Antonio Marchal, *University of Granada*
EDU03: Insights on lipid nanoparticles and exosomes

10:20 Cristian Smerdou, *CIMA, University of Navarra, Pamplona*
EDU04: Insights on self-amplifying RNAs and their delivery

09:00-10:45 · PARALLEL · Room Parallel B
EDUCATION SESSION 1b - Genetic diseases: there is more than one treatment strategy
Chairs: ***Gloria Gonzalez-Aseguinolaza,*** *CIMA, University of Navarra, Pamplona*

09:00 Welcome

09:05 Gloria Gonzalez-Aseguinolaza, *CIMA, University of Navarra, Pamplona*
EDU05: Gene addition vs gene editing strategies in liver-directed gene therapy

09:30 Virginia Haurigot
EDU06: Gene therapy for the sensory systems (vision and audition)

09:55 Jeff Chamberlain, *University of Washington School of Medicine*
EDU07: Gene addition vs gene editing strategies in muscular dystrophy

10:20 Paula Rio, *Ciemat Madrid*
EDU08: Gene addition vs. gene editing in Fanconi Anemia

10:45-11:15 · Pavilion 2
EDUCATION SESSION COFFEE BREAK

11:15-13:00 · PARALLEL · Room Parallel A
EDUCATION SESSION 2a - Gene and genome editing: from basics to applications
Chairs: ***Cristian Smerdou,*** *Cima Universidad de Navarra*

11:15 Toni Cathomen, *University of Freiburg*
EDU09: From Blunt Cuts to Fine Tweaks: Navigating the Therapeutic Genome Editing Toolbox

11:40 Angelo Lombardo, *SR-Tiget, Milan*
EDU10: Modifying the epigenome - novel strategies in gene therapy

12:05 Sandra Rodriguez, *Madrid*
EDU11: Gene editing as tool in cancer models

12:30 Francisco Martin, *GenYo, Granada*
EDU12: Improving living drugs by gene editing

11:15-13:00 · PARALLEL · Room Parallel B
EDUCATION SESSION 2b - Cell-based strategies to cure diseases
Chairs: ***Anne Galy,*** *ART-TG – Inserm US35*

11:15 Maria Eugenia Fernandez-Santos, *Madrid*
EDU13: Cell-based strategies in cardiovascular regeneration

11:40 Juan Roberto Rodriguez-Madoz, *CIMA, University of Navarra, Pamplona*
EDU14: CAR T and CAR NK cells in cancer and beyond

12:05 Aida Platero-Luengo, *IBiS, University Hospital Virgen del Rocío / CSIC / University of Seville*
EDU15: Partial Reprogramming: A Novel Approach to Combat Aging-related Decline

12:30 Ana Belen Muñoz Manchado, *Faculty of Medicine, University of Cadiz/INiBICA*
EDU16: Cell-based therapies in the Nervous System

09:30-11:30 · WORKSHOP · Room Parallel C
CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP 1
Chairs: **Alessandro Aiuti***, SR-Tiget, Milan &* **Juan Bueren***, Ciemat, Madrid*

09:30 Welcome and introduction

09:35 Barbara Bonamassa, *AIFA, Italy*
CTC01: Development and marketing authorization for ATMPs in the EU

09:55 Annette Kunkele, *JOIN4ATMP consortium*
CTC02: Results of the Join4ATMP survey on ATMP development and access

10:15 Zuzana Spacirova, Junta de Andalucia, *Granada*
CTC03: Understanding the pricing dynamics of advanced therapy medicinal products developed by academic developers in Spain

10:35 Beth White, *Orphan Therapeutics Accelerator*.
CT04: Not for profit models for accelerating development and commercialization of ultrarare orphan drugs

10:55 Roundtable and Q&A

11:30-11:45 · Pavilion 2
CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP COFFEE BREAK

11:45-13:00 · WORKSHOP · Room Parallel C
CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP 2
Chairs: **Alessandro Aiuti,** *SR-Tiget, Milan &* **Juan Bueren***, Ciemat, Madrid*

11:45 Christopher Baum, *Berlin Institute of Health at Charité, Berlin*
CTC05: National Approaches to Advance GCT in Europe: Potential for Synergies

12:05 Paschalia Koufokotsiou, *European commission*
CTC06: EU initiatives toward a sustainable ecosystem for ATMPs in Europe

12:25 Manuel Arellano, *Vice President of the Patient Organitzations Platfrom*
CTC07: Accessibility to ATMP: a patient perspective

12:45 Roundtable and Q&A

TUESDAY 7 OCTOBER

12:00-14:00 · Registration Foyer
REGISTRATION

14:00-16:30 · PLENARY · Auditorium
SESSION 1: ESGCT 2025 Opening: Gene Editing

Chairs: ***Alberto Auricchio****, Tigem, Naples;* ***Paula Rio,*** *Ciemat/CIBERER/IIS-Fundación Jiménez Díaz, Madrid;* ***Juan Jose Toledo-Aral,*** *Universidad de Sevilla*

14:00 Alberto Auricchio, *Tigem, Naples*
14:10 Paula Rio, *Ciemat/CIBERER/IIS-Fundación Jiménez Díaz, Madrid*
14:20 Juan Jose Toledo-Aral, *Universidad de Sevilla*
Welcome

14:30 Luigi Naldini, *SR-Tiget, Milan*
INV01: Paving the way to next-generation genetic engineering of hematopoiesis

15:00 Kiran Musunuru, *UPenn*
INV02: Developing and deploying N-of-1 gene-editing therapies

15:30 Omar Abudayyeh, *Harvard Medical School*
INV03: Programmable technologies for biology, genome editing, and mRNA therapies

16:00 Lukas Jeker, *University of Basel*
INV04: Molecular Cell Shielding on the Path to the Clinic

16:30-17:00 · Pavilion 2
COFFEE BREAK

17:00-19:30 · PARALLEL · Room Parallel A
SESSION 2a: Metabolic diseases I

Chairs: ***Brian Bigger,*** *University Of Edinburgh;* ***Nerea Zabaleta,*** *CIMA, University of Navarra*

17:00 Pasquale Piccolo, *TIGEM, Naples*
INV05: Overcoming the challenges of gene therapy in inborn error of metabolism with liver damage

17:30 George Diaz, *iECURE, Philadelphia, USA*
INV06: OTC-HOPE: The first in vivo, liver directed, AAV-mediated, gene insertion clinical trial in infants

18:00 Andrew Lin, *Lingyi Biotech*
OR001: Evaluation of Clinical Safety and Efficacy of LY-M001: A Phase I/II Trial of AAV8-Mediated Gene Therapy for Gaucher Disease Type I

18:15 Maria Ester Bernardo, *SR-Tiget, Milan*
OR002: Extensive metabolic detoxification and favorable clinical outcomes sustained through 5 years post-treatment in Hematopoietic Stem Cell Gene Therapy (OTL-203) for Mucopolysaccharidosis Type I-Hurler

18:30 Asmaa Naseem, *University College London, Institute of Child Health*
OR003: In vivo prenatal base editing for the treatment of Krabbe disease

18:45 Kate Mullany, *Children's Medical Research Institute, University of Sydney*
OR004: Exploring a single target tissue strategy for AAV-mediated gene transfer in Maple Syrup Urine Disease

19:00 Elena Barbon, *SR-Tiget, Milan*
OR005: Correction of glycogen storage disease type Ia by in vivo liver-directed lentiviral gene therapy in a mouse model

19:15 Shaun Wood, *University of Edinburgh*
OR006: Development of Heparan Sulfate Binding Peptides, fused to Iduronate-2-Sulfatase, for the Treatment of Mucopolysaccharidosis Type II

17:00-19:30 · PARALLEL · Room Parallel B
SESSION 2b: AAVs/Non integrative vectors: Technology

Chairs: ***Hildegard Büning,*** *Hannover Medical School;* ***Aravind Asokan,*** *Duke University*

17:00 Ben Deverman, *Broad Institute, Harvard*
INV07: Toward a one time epigenetic silencing treatment for prion disease with BBB crossing AAVs

17:30 Douglas Anderson, *University of Rochester Medical Center*
INV08: StitchR and CircuIR: From RNA Stitching Technologies to Therapeutics

18:00 Adrian Briggs, *Shape Therapeutics*
OR007: Engineered AAV5 capsid SHP-DB1 efficiently targets the NHP brain after intravenous injection and transduces >95% of neurons in the Parkinson's disease-critical substantia nigra

18:15 Stefano Parracino, *SR-Tiget, MIlan*
OR008: Covalent Antibody Conjugation to AAV9 for Targeted Gene Therapy of High-Risk Neuroblastoma

18:30 Shunsuke Iizuka, *JCR Pharmaceuticals*
OR009: Generation of a novel capsid engineering platform enhances CNS and muscle tropism of AAV vectors while reducing tropism to the liver

18:45 Lijia Ma, *Westlake University, China*
OR010: An AAV6 variant engineers human resting T cells in vivo

19:00 Cenfeng Chu, *OBiO Technology (Shanghai) Corp., Ltd.*
OR011: AAV-WM04, a Novel High-Efficiency Vector, Achieves Low-Dose Therapeutic Efficacy in Preclinical and Clinical Studies of *OTOF*-Related (DFNB9) Hearing Loss

19:15 Beatriz Serra, *University of Coimbra*
OR012: Validation of Systemic AAV-PHP.eB-Mediated Delivery of Cholesterol Hydroxylase CYP46A1 as a Non-Invasive Gene Therapy Approach for Spinocerebellar Ataxia Type 3

TUESDAY 7 OCTOBER

17:00-19:30 · PARALLEL · Room Parallel C

SESSION 2c: CNS & neurosensory I

Chairs: ***Ivana Trapani,*** *Telethon Institute of Genetics and Medicine;* ***Assumpcio Bosch,*** *Institut de Neurociències, Universitat Autònoma de Barcelona*

17:00 Bev Davidson, *University of Pennsylvania*
INV09: Improving on target gene therapies for the brain

17:30 Pietro Fratta, *Crick Institute, London*
INV10: Novel Gene Therapies for ALS and TDP-43 proteinopathies

18:00 Sergi Verdes, *Universitat Autonoma de Barcelona*
OR018: Targeting Skeletal Muscle to protect Motor Neurons: α-Klotho Gene Therapy acts through NMJ Signaling in ALS

18:15 Yuri Ciervo, *University of Padua*
OR016: Restoration of progranulin via microglia replacement with engineered hematopoietic stem cells corrects the phenotype of granulin-deficient mice

18:30 Sara Marco, *Universitat Autonoma de Barcelona*
OR013: Treatment of Sandhoff and Tay-Sachs diseases with an innovative gene therapy expressing a covalently linked HEXA protein

18:45 Hanen Khabou, *Sparing Vision*
OR014: Development of SPVN20, a gene therapy for cone reactivation in inherited retinal degeneration

19:00 Arjun Padmanabhan, *Tigem, Naples*
OR015: AAV-HITI for therapy of dominant retinitis pigmentosa

19:15 Stefano Espinoza, *Università Del Piemonte Orientale*
OR017: Multi-SINEUP: a novel RNA therapeutic approach for 22q11.2 microdeletion syndrome

17:00-19:30 · PARALLEL · Room Parallel D

SESSION 2d: Immunotherapy and CAR T cells I

Chairs: ***Chiara Magnani,*** *University of Zurich;* ***Waseem Qasim,*** *University College London*

17:00 Elena Sotillo, *Stanford University*
INV11: Combined silencing of MED12 and activation of IL2 by epigenetic editing enhances CAR T antitumor potency.

17:30 Philippe Parone, *EsoBiotec, Mont Saint Guibert*
INV12: In Vivo CAR-T Cell Therapy using Engineered Lentiviral Vectors: From Concept to Clinic

18:00 Malavika Sreekumar Nair, *Lund Stem Cell Centre, Lund University*
OR019: Epigenetic adjuvants for in vivo dendritic cell reprogramming

18:15 Jacek Lubelski, *Nanocell*
OR020: Novel non-viral DNA-based gene therapy vector for in vivo CAR T engineering: insights into CAR-T persistence and biodistribution

18:30 Susanna Cesarano, *SR-Tiget, Milan*
OR021: Development of antigen-specific Treg-based therapeutic products for Type 1 Diabetes

18:45 Eva García Veros, *Fundación para la Investigación Biomédica Hospital Universitario 12 de Octubre*
OR022: Development of dual-targeted STAb-T cells for cancer immunotherapy

19:00 Laura Cabizzosu, *Ludwig Institute for Cancer Research; UNIL; EPFL; CHUV - Lausanne*
OR023: Engineering Exhaustion-Resistant T Cells with IL-2 Variant (IL-2v) and CD40 Agonists to Enhance Antitumor Immunity

19:15 Daniela Cesana, *SR-Tiget, Milan*
OR024: Unveiling CAR-T Therapy Efficacy and Toxicity Through Cell-Free DNA Profiling

19:30-21:00 · Pavilion 2

WELCOME RECEPTION

WEDNESDAY 8 OCTOBER

08:00-08:30 · Registration Foyer
REGISTRATION

08:30-10:30 · PARALLEL · Room Parallel A
SESSION 3a: Infectious diseases / Vaccines

Chairs: ***Chantal Pichon,*** *INSERM ARNm;* ***Anne Galy,*** *ART-TG – Inserm US35*

08:30 Lisa Chakrabarti, *Institut Pasteur, Paris*
INV13: Development of lymphoid organ-on-chip models to evaluate mRNA vaccine immunogenicity

09:00 Robin Shattock, *Imperial College London*
INV14: Development of self-amplifying RNA for vaccines and therapeutics

Winner of the ESGCT breakthrough of the year award supported by the Fresenius Foundation
09:30 Nerea Zabaleta, *CIMA, University of Navarra*
INV15: Immunogenic rAAVs as genetic vaccines

10:00 Rut Mora Buch, *Blood and Tissue Bank, Barcelona*
OR025: BK virus-specific T cell response associated with HLA genotypes, RhD status, and CMV or EBV serostatus in healthy donors for optimized cell therapy

10:15 Stefanie Gawletta, *TronGmbH*
OR026: Development of a trans-amplifying RNA that conserves its performance upon N1-methylpseudouridine modification

08:30-10:30 · PARALLEL · Room Parallel B
SESSION 3b: Virotherapy and cancer gene therapy

Chairs: ***Marta Alonso,*** *Universidad de Navarra;* ***Dirk Nettlebeck,*** *German Cancer Research Center Heidelberg*

08:30 Ramon Alemany, *Catalan Institute of Oncology, IDIBELL*
INV16: Cancer Virotherapy with Armed Oncolytic Adenoviruses

09:00 Alan Melcher, *Institute of Cancer Research, London*
INV17: T cell receptor-antigen engagement dynamics with oncolytic virotherapy defines novel subsets of anti-tumour CD8 cells

09:30 Kate Friesen, *University of Oxford*
OR030: Exosomes as a stromal-targeting extension of oncolytic virus therapeutics

09:45 Alvaro Morales-Molina, *Instituto de Salud Carlos III, Madrid*
OR028: Phase Ib clinical trial assessing safety, tolerability, and preliminary efficacy of AloCELYVIR (allogeneic mesenchymal cells + oncolytic adenovirus) in pediatric patients with recurrent/progressive medulloblastoma

10:00 Salvatore Russo, *University of Helsinki*
OR029: Effect of Extracellular vesicles in remodeling the Tumor Micro Environment by DNMT1 downregulation for enhanced cancer immunotherapy

08:30-10:30 · PARALLEL · Room Parallel C
SESSION 3c: Nanomedicines for gene delivery: From research to clinical translation

Chairs: ***Hélder Santos,*** *University Medical Center Groningen, University of Groningen;* ***Maria Alonso,*** *University of Santiago de Compostella*

08:30 Willem Mulder, *Radboud Universiteit*
INV18: Nature-inspired mRNA delivery to immune cells

09:00 Maria Kavallaris, *UNSW Sydney*
INV19: Targeting high-risk paediatric cancer on a tiny scale

09:30 Liliana Mendonça, *Centre for Neuroscience and Cell Biology - University of Coimbra*
OR031: Differentiation-inducing drug encapsulated in brain-targeted liposomes with enhanced brain delivery increased human iPSC-derived neuroepithelial stem cell differentiation into neurons

09:45 Guillem Pascual Pasto, *The Children Hospital of Philadelphia*
OR032: Restoration of Rb tumor suppressor function in retinoblastoma via GPC2-targeted RB1 mRNA lipid nanoparticles

10:00 Eline Van Diest, *UMC Utrecht*
OR033: Specificity and biodistribution of a novel T-cell targeted LNP designed for efficient and stable CAR T generation *in vivo*

10:15 Go Sugahara, *PhoenixBio Co. Ltd*
OR034: Species-specific gene expression manipulation in humanized livers of chimeric mice via siRNA-encapsulated lipid nanoparticle treatment

08:30-10:30 · PARALLEL · Room Parallel D
SESSION 3d: Lentiviral vectors / Integrative vectors

Chairs: ***Christian Brendel,*** *Harvard Medical School;* ***Axel Schambach,*** *Hannover Medical School*

08:30 Mario Leonardo Squadrito, *SR-TIGET, Milan*
INV20: Engineered Lentiviral Vectors for the Therapeutic Targeting of Liver Metastases

09:00 Cecilia Cotta-Ramusino, *Tessera Therapeutics*
INV21: Writing DNA with RNA: Genome Engineering by Target-Primed Reverse Transcription

09:30 Chiara Bresesti, *SR-Tiget, Milan*
OR035: Comet LV: a lentiviral vector-based mRNA co-packaging technology for enhanced ex vivo and in vivo gene therapy

09:45 Nathan Jeffreys, *Paediatric BMT and gene therapy department, Royal Manchester Children's Hospital*
OR036: Ex-vivo modification of autologous CD34+ HSPCs using a CD11B-directed lentiviral vector encoding APOEII-tagged human IDS leads to supraphysiological enzyme activity and early biochemical correction of neuropathic mucopolysaccharidosis type II

10:00 Susana Navarro Ordonez, *CIEMAT / CIBERER / IIS-Fundación Jimenez Diaz*
OR037: Toward Clinical Translation of Lentiviral Gene Therapy for Diamond-Blackfan Anemia: Preclinical Studies Targeting RPS19

10:15 Francesco Gazzo, *SR-Tiget, Milan*
OR038: Oncogene activation by lentiviral vectors drives senescence-linked clonal haematopoiesis and mutation accumulation in HSPC gene therapy

WEDNESDAY 8 OCTOBER

10:30-11:00 · Pavilion 2
COFFEE BREAK

11:00-13:00 · PLENARY · Auditorium
SESSION 4: Cancer gene therapy

Chairs: ***Francisco Martin,*** *GENYO - Universidad de Granada;* ***Bernhard Gentner,*** *Centre Hospitalier Universitaire Vaudois (CHUV)*

11:00 Sonia Guedan, *IDIBAPS, Barcelona*
INV22: Next-Generation CAR-T Cells: Overcoming Resistance Toward Clinical Translation

11:30 Katy Rezvani, *MD Anderson*
INV23: Optimizing NK Cell Engineering for Therapeutic Efficacy

12:00 Luis Álvarez Vallina, *CNIO Madrid*
INV24: Engineered STAb-T cells secreting anti-BCMA T cell engagers control multiple myeloma and promote immune memory in vivo.

12:30 Vincenzo Cerullo, *University of Helsinki*
INV25: PeptiCRAd: From Lab Discovery to the Clinic – A Highly Customizable Platform for Personalized Cancer Vaccines

13:00-14:00 · Pavilion 2
LUNCH

13:30-14:30 · Room Parallel A
LUNCHTIME SYMPOSIUM: PlasmidFactory - Virus-free genetic engineering using Minicircles for scalable & effective cell therapies

Chairs: ***Michael Hudecek,*** *Universität Würzburg;* ***Dirk Winnemöller,*** *PlasmidFactory GmbH, Bielefeld*

13:30 Dirk Winnemöller, *PlasmidFactory GmbH, Bielefeld*
Welcome and brief introduction on PlasmidFactory's Minicircle

13:40 Michael Hudecek, *Universität Würzburg*
Brief introduction on Minicircle-based CAR-T cells in clinical applications

13:50 Zoltán Ivics, *Universität Leipzig*
Enhanced efficiency of Sleeping Beauty transposon engineering of therapeutic cells by Minicircle vectors

14:00 Volker Lennerz, *TheryCell GmbH, Berlin*
Non-viral manufacturing of tumor-specific TCR-T cells for immunotherapy of multiple advanced solid cancers

14:10 Juan R. Rodriguez-Madoz, *Universidad de Navarra*
Transposon-based non-viral vectors for improved CAR-T therapies

14:20 Ulf Grawunder, *T-CurX GmbH, Würzburg*
Promises and challenges of in vivo CAR-T therapies

All speakers are available for Q&A
Or visit us at booth B21/B22 for your individual questions

13:30-14:30 · Room Parallel B
LUNCHTIME SYMPOSIUM: ERC-EIC: From Frontier Research to Innovation: ERC's and EIC's funding opportunities

Chairs: ***Orsolya Symmons,*** *EIC;* ***Janka Mátrai,*** *ERC*

13:30 Tina Lebar, *National Institute of Chemistry &* **Raffaella Di Micco**, *SR-Tiget, Milan*

13:30-14:30 · Room Parallel D
LUNCHTIME SYMPOSIUM: Sartorius - Walking the Tightrope | Balancing Process Optimization & Cost Implications to Improve CGT Accessibility

Chair: ***Hanna P. Lesch,*** *Independent Expert - Advanced Therapies / Chair of ESGCT Manufacturing Committee / Adj. Prof. Biomedicine and Bioprocessing, University of Eastern Finland*

13:30 David Ede, *Segment Technology Manager, Advanced Therapy Solutions*
14:00 Behnam Partopour, *Principal Scientist, Advanced Bioprocessing*

14:00-15:30 · Fibes 2
POSTER SESSION
Uneven Posters P0001, P0003, P0005 etc

15:30-17:30 · PLENARY · Room Auditorium
SESSION 5: In vivo gene therapy

Chairs: ***Gloria Gonzalez-Aseguinolaza,*** *CIMA, University of Navarra, Pamplona;* ***Pasquale Piccolo***, *Tigem Naples*

15:30 Jeffrey Chamberlain, *University of Washington*
INV26: Next generation gene therapies for DMD

16:00 Ellen Reisinger, *University of Tübingen*
INV27: OTOF – from basic research to gene therapy for hearing impairment

16:30 Birgit Schultes, *Intellia Therapeutics*
INV28: In vivo CRISPR/Cas9 gene editing for ATTR amyloidosis and HAE - how close are we to getting these therapies to the market?

17:00 Terry Flotte, *University of Massachusetts*
INV29: Gene Therapy for Tay-Sachs and Sandhoff Diseas

WEDNESDAY 8 OCTOBER

17:30-18:00 · Pavilion 2
COFFEE BREAK

18:00-19:30 · PARALLEL · Room Parallel A
SESSION 6a: Manufacturing I: Delivery technologies

Chairs: ***Ana Coroadinha,*** *iBET;* ***Matthias Hebben,*** *Complement Therapeutics Ltd*

18:00 Cesar Trigueros, *Viralgen*
INV30: A Strategic Platform Roadmap for Commercializing Gene Therapy

18:30 Virginia Fusco, *Tigem, Naples*
OR040: Engineering a stable cell line for AAV production through synthetic biology.

18:50 Tarik Hadi, *Takara Bio Europe*
OR041: Maximizing MSC-EV Yield with Preserved Quality Using a Xeno-Free, Defined Formulation

19:10 Marco Radukic, *Bielefeld University*
OR043: Multivariate toolchain for analysis of emerging AAV critical quality attributes

18:00-19:30 · PARALLEL · Room Parallel B
SESSION 6b: Cardiovascular & muscular diseases

Chairs: ***Thierry VandenDriessche****, VUBrussels;* ***Fulvio Mavilio****, Orchard Therapeutics*

18:00 Wolfram Zimmermann, *Goettingen University*
INV31: Engineered heart muscle allografts for heart repair in primates and humans

18:30 Didier Stainier, *Max Planck Institute for Heart and Lung Research, Bad Neuheim*
INV32: Transcriptional adaptation, an RNA-based mechanism of genetic compensation

19:00 Seppo Yla Herttuala, *University of Eastern Finland*
OR044: Adenoviral vascular endothelial growth factor-DΔNΔC gene therapy improves clinical symptoms, exercise tolerance and health-related quality of life in refractory angina pectoris patients. ReGenHeart Phase 2 trial

19:15 Han Qiu, *Accuredit Therapeutics*
OR045: Efficacy and safety of ART002, a single-dose gene editing therapy, in patients with severe heterozygous familial hypercholesterolemia

18:00-19:30 · PARALLEL · Room Parallel C
SESSION 6c: CNS & neurosensory II

Chairs: ***Robin Ali,*** *Kings College London;* ***Ellen Reisinger,*** *University of Tübingen*

18:00 Paul Yang, *Casey Eye Instituite, Portland*
INV33: Safety and efficacy of a phase 1/2 clinical trial for AAV5-mediated retinal gene augmentation of GUCY2D in Leber congenital amaurosis 1

18:30 Steven Gray, *UT Southwestern*
INV34: Utilizing and expanding AAV platform approaches to treat neurological diseases

19:00 Françoise Piguet, *GENOV - Paris Brain Institute*
OR046: Combine intravenous gene therapy overexpressing CYP46A1 and BBB opening can rescue Parkinson disease in a NHP induced PD model

19:15 Jiake Zhong, *Fudan University*
OR047: Gene therapy vs. cochlear implantation in restoring hearing function and speech perception for congenital deafness individuals

18:00-19:30 · PARALLEL · Room Parallel D
SESSION 6d: Non viral vectors / Nanotechnology

Chairs: ***Zoltan Ivics,*** *University of Leipzig/Fraunhofer Institute IZI;* ***Willem Mulder,*** *Radboud Universiteit*

18:00 Helder Almeida Santos, *University Medical Center Groningen*
INV35: Beta-Glucan nanoformulations for cardiac RNA-based therapies

18:30 Maria Alonso, *University of Santiago de Compostella*
INV36: Lipid-Polymer Hybrid (LPH) Nanoparticles: Next-Generation Platforms for RNA Therapeutics

19:00 Jesus Beltran, *DNAmic Biotech Valencia, The Scripps Research Institute, La Jolla, CA - MIT, Cambridge, MA - University of California, San Diego*
OR048: Development of a fully human-based delivery platform for in vivo cell-targeted therapies

19:15 Sander Kooijmans, *University Medical Center, Utrecht*
OR049: A novel siRNA backbone improves siRNA delivery by biological vectors

THURSDAY 9 OCTOBER

08:00-08:30 · Registration Foyer
REGISTRATION

08:30-10:30 · PARALLEL · Room Parallel A
SESSION 7a: Immune responses to gene therapy

Chairs: ***Gwladys Gernoux,*** *TaRGeT, Nantes University, Inserm;* ***Carmen Unzu,*** *CIMA, University of Navarra, Pamplona*

08:30 Giuseppe Ronzitti, *Genethon*
INV37: Harnessing the potential of Immunoglobulin G degrading enzymes (Ide) for the treatment of AAV-seropositive patients

09:00 Oumeya Adjali, *University of Nantes*
INV38: Navigating Adaptive Immune Responses to AAV Vectors: Implications for Gene Therapy

09:30 Rebecca Xicluna, *F.Hoffmann-La Roche Ltd*
OR050: Transient blockade of the CD28/B7 costimulatory pathway allows AAV readministration by suppressing AAV vector and transgene immunity

09:45 Carla Llinares-Simon, *CIMA, Centro de Investigación Médica Avanzada, University of Navarra, Pamplona*
OR051: Characterization of an immunotolerant peptide display for the modulation of the anti-AAV immune response

10:00 Jean-Philippe Combal, *Vivet Therapeutics*
OR052: VTX-PID (imlifidase) Mediated Optimal Depletion of Anti-AAV3B Neutralizing Antibodies in Human Subjects allows to Expand Patient Eligibility for future AAV-Based Gene Therapies : Results of a dose ranging and PK/PD modeling approach

10:15 Isabella Ramella Gal, *iBET - Institute of Experimental Biology and Technology*
OR053: A human 3D liver model to unravel rAAV-driven innate immune responses

08:30-10:30 · PARALLEL · Room Parallel B
SESSION 7b: Hematopoietic & PID I

Chairs: ***Don Kohn****, UCLA;* ***Giorgia Santilli****, University College London*

08:30 Anna Villa, *SR-Tiget*
INV39: HDR mediated Gene Editing in Hematopoietic Stem and progenitor Cells to cure RAG1 deficiency

09:00 Sung-Yun Pai, *NIH - RECORDING*
INV40: Current status of gene therapy for X-linked severe combined immunodeficiency

09:30 Denise Klatt, *Dana Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School*
OR054: Engineered alpha retroviral like particles for in vivo GT

09:45 Daniele Canarutto, *SR-Tiget, Milan*
OR055: Single-cell DNA sequencing provides a comprehensive assessment of genomic editing outcomes of gene-corrected CD4 T-cells for treating Hyper IgM1 supporting the start of clinical trial

10:00 Annalisa Santini, *Imagine Institute, Paris*
OR056: Correction of the SCD mutation with a safe base-editing strategy characterized by high specificity and low toxicity

10:15 Karin Pike Overzet, *Leiden University Medical Center*
OR057: Clinical trial update: Autologous hematopoietic stem cell-based lentiviral gene therapy for Recombination-activating gene-1 severe combined immunodeficiency (RAG1-SCID)

08:30-10:30 · PARALLEL · Room Parallel C
SESSION 7c: Gene Editing I: Ex vivo applications

Chairs: ***Samuele Ferrari,*** *Ospedale San Raffaele, Milan;* ***Marianne Carlon,*** *KU Leuven*

08:30 Marcello Maresca, *Astra Zeneca*
INV41: In vivo Genome Editing with SpOT-On precision

09:00 Toni Cathomen, *University of Freiburg*
INV42: Under the Lens: Unpredictable Off-Target and Structural Effects in Genome Editing

09:30 Alessandra Weber, *SR-Tiget, Milan*
OR058: A universal and versatile platform for safe and efficient targeted integration of therapeutic genes in hematopoietic stem cells

09:45 Pietro Rigoni, *University of Padua*
OR059: A novel and empowered TARGETED Gene Addition Approach at a Relevant Microglia Locus for the Treatment of X-linked Adrenoleukodystrophy -- Mechanistic Insights

10:00 Manuel Palacios Perez, *Ciemat / Ciberer / IIS-FJD*
OR060: Optimized therapeutic homologous recombination in RPL5-deficient Diamond-Blackfan anemia hematopoietic stem and progenitor cells

10:15 Giandomenico Turchiano, *University College London*
OR061: Longitudinal Tracking of Chromosomal Stability in Mice After Gene Editing

THURSDAY 9 OCTOBER

08:30-10:30 · PARALLEL · Room Parallel D

SESSION 7d: Skin, pulmonary & skeletal diseases

Chairs: ***Uta Griesenbach,*** *Imperial College London;* ***Fernando Larcher,*** *Department of Bioengineering, UC3M, Madrid*

08:30 Matthias Titeux, *Institut Imagine*
INV43: Splice modulation strategies using antisense oligonucleotides for Recessive Dystrophic Epidermolysis Bullosa

09:00 Fleur van Dijk, *London North West Healthcare University NHS Trust*
INV44: Repair of fragile tissues: a clinical genetic perspective on the issues

09:30 Daniel Anderson, *MIT*
INV45: Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing

10:00 Annahita Keravala, *Genascence*
OR062: Twelve-month safety, tolerability, and pharmacodynamics data from DONATELLO: A Phase 1b trial of locally administered AAV-mediated anti-interluekin-1 gene therapy in subjects with knee osteoarthritis

10:15 Sebastian Aguirre Kozlouski, *Carbon biosciences*
OR063: CGT-001; A recombinant human bocavirus vector overcoming challenges observed with existing gene therapy approaches to efficiently deliver therapeutic levels of CFTR

10:30-11:00 · Pavilion 2

COFFEE BREAK

11:00-13:15 · PLENARY · Auditorium

SESSION 8: Presidential symposium

Chairs: ***Alberto Auricchio,*** *Tigem, Naples;* ***Paula Rio,*** *Ciemat/CIBERER/IIS-Fundación Jiménez Díaz, Madrid;* ***JuanJose Toledo Aral,*** *Universidad de Sevilla*

11:00 Alberto Auricchio, *Tigem, Naples*
INV46: Presidential address

11:15 Juan Carlos Ispizua Belmonte, *Altos Labs, San Diego Institute of Science*
INV47: Restoring Cell Health and Reversing Disease by Cellular Rejuvenation

11:45 OUTSTANDING ACHIEVEMENT AWARD
Juan Bueren, *Ciemat Madrid*
INV48: Gene Therapy for patients with Fanconi anemia: A stem cell disease

12:15 YOUNG INVESTIGATOR AWARD
Gabriele Casirati, *Boston Children's Hospital/Dana Farber Cancer Institute*
OR064: Epitope engineering to enhance cancer immunotherapy, gene therapy and beyond

12:30 CAREER PROGRESSION AWARD
Michela Milani, *SR-Tiget, Milan*
OR065: In vivo lentiviral vector gene transfer into hematopoietic stem and progenitor cells

12:45 Ivana Trapani, *TIGEM, Naples*
OR066: Advancing AAV-based gene therapy for inherited retinal disorders due to mutations in large genes.

Presentation of the ESGCT Breakthrough of the Year Award supported by the Fresenius Foundation

13:00-13:15 · Auditorium

ESGCT AGM

13:00-14:00 · Pavilion 2

LUNCH

13:30-14:30 · Room Parallel A

LUNCHTIME SYMPOSIUM: Manuscript submission, review & publishing process from a journal's perspective and alternative career paths in journal editing and scientific publishing

13:30 Kevin Davies, *The CRISPR Journal, GEN Biotechnology*

13:45 Joshua Varley-Reeves, *eBioMedicine, Lancet*

14:00 Thomas Gallagher, *Human Gene Therapy*

Panel discusion

13:30-14:30 · Room Parallel B

LUNCHTIME SYMPOSIUM: Merck - To 1000L and beyond: Innovating Upstream AAV manufacturing for the next wave of gene therapies

13:30 Fletcher Malcom, *Director of Strategy, Product and Business Development*

13:30-14:30 · Room Parallel D

LUNCHTIME SYMPOSIUM: MaxCyte - Scalable, Non-Viral Technologies in Action from Concept to Clinic

Chairs: ***Alicia Roig-Merino,*** *Regional Manager, FAS Field Support - Europe, MaxCyte Inc.*

13:30 Elena Stoyanova, PhD, *Principal Scientist, Touchlight - London, United Kingdom*

13:50 Richard Harbottle, PhD, *Head of DNA Vector Research, German Cancer Research Centre (DKFZ) - Heidelberg, Germany*

14:10 Begoña Diez Cabezas, PhD, *Researcher CIEMAT/CIBERER/IIS-FJDager at CliniStem (GMP facility) - Madrid, Spain*

14:00-15:30 · Fibes 2

POSTER SESSION
Even Poster numbers (P0002, P0004, P0006 etc)

THURSDAY 9 OCTOBER

15:30-17:30 · PARALLEL · Room Parallel A

SESSION 9a: Gene Editing II, Ex vivo applications

Chairs: ***Angelo Lombardo,*** *SR-Tiget, Milan;* ***Sean Chen,*** *Mammoth Biosciences, Brisbane*

15:30 Marianne Carlon, *KU Leuven*
INV49: Innovative in vitro and ex vivo organoid and lung models support base and prime editing studies to re-write drug-refractory CFTR mutations

16:00 Melissa Bonner, *nChroma*
INV50: In Vivo Epigenetic Silencing is Poised to DLVR

16:30 Sean Chen, *Mammoth Biosiences*
OR067: Highly efficient lowering of serum APOC3 and triglyceride levels using an engineered CasPhi nuclease in NHPs and a mouse model of severe hypertriglyceridemia as basis for treatment of persistent chylomicronemia in humans

16:45 Yannick Doyon, *Laval University*
OR068: In vivo editing of the transferrin locus for systemic correction of lysosomal storage diseases

17:00 Linda Burkly, *Editas Medicine, Inc*
OR069: A transformative LDL cholesterol--lowering in vivo CRISPR gene editing medicine that functionally upregulates LDLR in mice and non-human primates

17:15 Chiara Simoni, *SR-Tiget, Milan*
OR070: Development of in vivo genome editing for the treatment of progressive familial intrahepatic cholestasis type 2

15:30-17:30 · PARALLEL · Room Parallel B

SESSION 9b: AAVs/Non integrative vectors II : Mechanism & biology

Chairs: ***Isabelle Richard,*** *Genethon;* ***Dirk Grimm,*** *University of Heidelberg*

15:30 Li-An Brown, *University College London*
INV51: Insights into AAV-related hepatitis

16:00 Aravind Asokan, *Duke University School of Medicine*
INV52: Unlocking AAV Biology to Engineer Vector Safety

16:30 Pedram Moeini, *Center for Applied Medical Research (CIMA), University of Navarra, Pamplona*
OR071: High-dose AAV9.CBA.SMN1 administration triggers liver damage, cell-type remodeling and UPR activation in non-human primates but not in adult rodents

16:45 Izabela Kraszewska, *King's College London*
OR072: Small non-coding RNA adjuvants that improve cardiac transduction by AAV vectors

17:00 Saqlain Suleman, *Anglia Ruskin University*
OR073: AAV tethering to the genome is associated with transcription factor binding sites found in genes that favour viral integration

17:15 Ines Akrouf, *Myologie Institute, Sorbonne University, Paris*
OR074: Efficacy of AAV vectors and optimization of therapies in mouse models of Duchenne muscular dystrophy and Dynamin 2-related dominant centronuclear myopathy

15:30-17:30 · PARALLEL · Room Parallel C

SESSION 9c: Immunotherapy & CAR T cells II

Chairs: ***Karl Petri,*** *University Hospital Würzburg;* ***Dimitrios Wagner****, Baylor College of Medicine, Houston*

15:30 Caroline Arber, *UNIL, Lausanne*
INV53: Leveraging protein design for CAR-T cell engineering

16:00 Yvonne Chen, *UCLA, Los Angeles*
INV54: Engineering Multi-Pronged CAR-T Cell Therapy for Cancer

16:30 Alba Rodriguez-Garcia, *IDIBAPS, Barcelona*
OR075: Lipid nanoparticle-mediated DNA delivery triggers CD8+ T cell-dependent antitumor immunity and enhances PD-L1 blockade in mouse models of hepatocellular carcinoma

16:45 David Rawlings, *University of Washington*
OR076: CD19 CAR-Engineered Regulatory T Cells Suppress T And B Cell Driven Autoimmunity and Confer Durable Therapeutic Benefit in Mouse Models Of SLE

17:00 Paula Barbao, *IDIBAPS, Barcelona*
OR077: In vivo-based CRISPR screen identifies ZC3H12C as a mediator of CAR-T cell dysfunction in solid tumors

17:15 Eliana Ruggiero, *IRCCS San Raffaele Scientific Institute, Milan*
OR078: Acute myeloid leukemia eradication with TCR-redirected T cells targeting Cathepsin G antigen

THURSDAY 9 OCTOBER

15:30-17:30 · PARALLEL · Room Parallel D

SESSION 9d: Accessibility of gene therapy

Chairs: ***Alessandro Aiuti,*** *SR-Tiget, Milan;* ***Claire Booth,*** *UCL Institute of Child Health*

15:30 Terry Flotte, *University of Massachusetts*
INV55: Overcoming Barriers to Commercially Pre-Viable Gene and Cell Therapies for Rare an Ultra-Rare Diseases

15:55 Karen Oprych, *Great Ormond Street Hospital, London*
INV56: Not-for-profit manufacture and supply of economically challenging ATMPs for rare diseases.

16:20 Janet Glassford, *MHRA*
INV57: MHRA's progressive strategy for rare disease

16:45 recording of Jennifer Adair, *University of Massachusetts*
INV58:

17:10 Stefano Benvenuti, *Telethon Foundation*
INV59: Fondazione Telethon current strategies and future perspectives on patient access to gene therapy for ultra-rare disease

17:30-18:00 · Pavilion 2

COFFEE BREAK

18:00-19:30 · PARALLEL · Room Parallel A

SESSION 10a: Manufacturing II: Cell therapies

Chairs: ***Hanna Lesch,*** *Independent Expert - Advanced Therapies;* ***Ander Izeta,*** *Biogipuzkoa Health Research Institute, San Sebastian*

18:00 Jan Schrooten, *Antleron*
INV60: Digital twin-driven fixed-bed bioreactor platform for scalable manufacturing of viral vectors and virus-like particles: translation from lab towards patient

18:20 Ruiz Astigarraga, *Galapagos BV*
INV61: The Galapagos Decentralized Cell Therapy Manufacturing Platform

18:40 Justin Lievense, *BioTherapeutics Unit, Netherlands Cancer Institute*
OR079: Platform-based manufacturing of tumor-infiltrating lymphocyte medicinal products for treating solid tumors

18:55 Lasse Saaby, *Bioneer A/S*
OR080: Mapping thermophysical dynamics of cell-based formulations during cryopreservation

19:10 Paula Marcus, *Cytiva*
OR081: Large-scale transfection of T cells in a Xuri™ cell expansion system W25 using LNPs

18:00-19:30 · PARALLEL · Room Parallel B

SESSION 10b: Bleeding disorders

Chairs: ***Thierry VandenDriessche****, VUBrussels,* ***Antonia Follenzi****, Università del Piemonte Orientale*

18:00 Lindsey George, *University of Pennsylvania School of Medicine*
INV62: Next Generation Hemophilia A Gene Therapy

18:30 Graham Foster, *Queens University, London*
INV63: Liver related problems with gene therapy – what can we do to mitigate them?

19:00 Paula Sureda Horrach, *Tigem, Naples*
OR082: Homology-independent targeted integration in a humanised mouse model of Haemophilia A

19:15 Nathalie Jansen, *CSL Behring*
OR083: Sustained disease correction is achieved without long-term vector-related liver toxicity four years after etranacogene dezaparvovec hemophilia gene therapy both in individuals with and without early transaminitis

18:00-19:30 · PARALLEL · Room Parallel C

SESSION 10c: National initiatives in gene & cell therapy

Chairs: ***Zoltan Ivics,*** *University of Leipzig/Fraunhofer Institute IZI*

5 minutes presentations on the initiatves in different countries
INV64: Chrstian Gallus, *Berlin Institute of Health at Charité, Berlin*

INV65: Stefano Benvenuti, *Telethon Foundation, Milan*

INV66: Manel Juan, *Coordinator of the TERAVplus network, Spain*

INV67: Emmanuel Dequier, *Minsitry of Research, France*

INV68: Jeannette Evans, Catapult, *UK*

Panel discussion & round table

THURSDAY 9 OCTOBER

18:00-19:30 · PARALLEL · Room Parallel D

SESSION 10d: Late breaking news from the clinic

Chairs: ***Fatima Bosch*** *UAB, Barcelona,* ***Nathalie Cartier***

18:00 Olivier Danos, *RegenX*
INV70: RGX-202, an investigational gene therapy for the treatment of Duchenne muscular dystrophy: interim clinical data

18:30 Francesco Testa *Università degli studi della Campania Luigi Vanvitelli*
INV71: LUCE: Phase I/II Trial of Dual AAV Gene Therapy for Retinitis Pigmentosa in Usher Syndrome Type 1B

19:00 Samik Basu, *Cabaletta Bio*
OR084: RESET-PV: Initial clinical and translational data evaluating rese-cel (resecabtagene autoleucel), an autologous 4-1BBz CD19-CAR T cell therapy, without preconditioning, in pemphigus vulgaris

19:15 Arnaud Valent, *Genethon*
OR085: GNT0004, Genethon's AAV-based gene therapy for Duchenne muscular dystrophy: long-term follow-up of ambulatory boys enrolled in the dose-escalation phase of GNT-016-MDYF

19:30 BUSES DEPART TO Molecular Mingle
20:30 · Hazienda las Azaharas

MOLECULAR MINGLE

FRIDAY 10 OCTOBER

08:30-09:00 · Registration Foyer
REGISTRATION

09:00-10:30 · PARALLEL · Room Parallel A
SESSION 11a: Metabolic diseases II

Chairs: ***Giuseppe Ronzitti,*** *Genethon;* ***Nicola Brunetti-Pieri,*** *Tigem, Naples*

09:00 Paul Gissen, *University College London*
INV72: AAV-mediated gene therapy for OTC deficiency

09:30 Rob Wynn, *Manchester University*
INV73: Hematopoietic stem cell and stem cell gene therapy in metabolic diseases

10:00 Jeremy Do Cao, *Antoine-Béclère Hospital, APHP*
OR086: Overcoming AAV8 Immunity: First Seropositive Crigler-Najjar Patient Treated with GNT0003 Following Imlifidase Pretreatment (GNT-018-IDES clinical trial)

10:15 Maria Battipaglia, *Tigem, Naples*
OR087: Modulation of TRPML1/TFEB pathway for the treatment of Wilson Disease

09:00-10:30 · PARALLEL · Room Parallel B
SESSION 11b: AAVs/Non integrative vectors III: Translation

Chairs: ***Ivana Trapani,*** *Tigem, Naples;* ***Gloria Gonzalez- Aseguinolaza,*** *CIMA, University of Navarra*

09:00 James Wilson, *Gemma Bio*
INV74: Lessons learned from 35 years of in vivo gene therapy research

09:30 Jonathan Schwartz, *Rocket Pharma*
INV75: Transformative Treatment of Devastating Cardiomyopathies: Safety and Efficacy in AAV Clinical Development of RP-A501 (Danon Disease) and RP-A601 (PKP2-ACM)

10:00 Lorenzo D'Antiga, *Hospital Papa Giovanni XXIII, Bergamo*
OR088: Durability of AAV-based gene therapy in patients with Crigler Najjar syndrome at 4 years of follow up after dosing: the GNT-012-CRIG Study

10:15 Andrea Perez Iturralde, *Children's Medical Research Institute, Australia*
OR089: Advancing AAV9-mediated gene therapy for CTNNB1 Syndrome: safety, efficacy, and clinical translation

09:00-10:30 · PARALLEL · Room Parallel C
SESSION 11c: Immunotherapy & CAR T cells III

Chairs: ***Eliana Ruggiero,*** *IRCCS Ospedale San Raffaele, Milan;* ***Sonia Guedan,*** *IDIBAPS, Barcelona*

09:00 Jay Daniels *Northwestern, Chicago*
INV76: Leveraging T cell mutations for enhanced T cell therapies

09:30 Claudia Rossig, *University Children's Hospital, Munster*
INV77: Cytokine-enhanced CAR T cells for solid tumors

10:00 Angela Covo-Vergara, *Center for Applied Medical Research (CIMA), Pamplona*
OR090: Self-amplifying RNA vaccines to improve the performance of CAR-T cells in solid tumors

10:15 Chiara Magnani, *University of Zurich*
OR091: Acquisition of an immunosuppressive microenvironment after CD19 CAR-T therapy is associated with T-cell dysfunction and resistance

10:30-11:00 · Pavilion 2
COFFEE BREAK

11:00-13:00 · PARALLEL · Room Parallel A
SESSION 12a: Regulatory requirements for clinical trial applications

Chairs: ***Sol Ruiz,*** *AEMPS, Madrid;* ***Klaus Cichutek***

11:00 Klaus Cichutek,
INV82: ATMP achievements and role of regulators

11:20 Matthias Renner, *Paul Ehrlich Institute, Langen*
INV83: First-in-human clinical trials: Regulatory considerations on manufacturing and control

11:40 Janet Glassford, *MHRA*
INV84: Advancements at the MHRA to support ATMP development

12:00 Paschalia Koufokotsiou, *European Commission*
INV85: Revision of the EU General Pharmaceutical Legislation: Impact on Advanced Therapy Medicinal Products (ATMPs).

12:20 Yeowon Sohn, *Korea Food and Drug Administration*
INV86: Comparative analysis of regulations and studies on stem cell therapies: focusing on induced pluripotent stem cell (iPSC)-based treatments

12:40 Panel Discussion

FRIDAY 10 OCTOBER

11:00-13:00 · PARALLEL · Room Parallel B

SESSION 12b: Disease models

Chairs: ***Maurilio Sampaolesi,*** *KU Leuven;* ***Rik Gijsbers,*** *KU Leuven*

11:00 Simão José Teixeira da Rocha, *University of Lisbon*
INV87: Stem-cell modeling of cerebellar dysfunction in Angelman syndrome

11:30 Xavier Trepat, *Institute of bioengineering of Catalunia, Barcelona*
INV88: Engineering the immunocompetent tumour ecosystem

12:00 Clémence Lièvre, *Nantes Université, CHU Nantes, INSERM, TaRGeT, (Translational Research in Gene Therapy - UMR 1089*
OR092: Engineered Muscle Tissues with enhanced maturation for the evaluation of muscle-tropic AAV vectors

12:15 Akshatha Shivaraj, *Hannover Medical School*
OR093: Deciphering the role of instructive transcriptional regulators on the hemogenic endothelium during human embryonic hematopoiesis using induced pluripotent stem cells

12:30 Patrizia Tornabene, *Cincinnati Children's Hospital Medical Center*
OR094: Modeling Cystic Fibrosis pancreatic complications in iPSC-derived organoids

12:45 Maria Herrera, *University of Seville*
OR095: Amyotrophic Lateral Sclerosis (ALS) brain organoids as a tool for the development of nano-gene therapies

11:00-13:00 · PARALLEL · Room Parallel C

SESSION 12c: Gene Editing III: Technology & applications

Chairs: ***Paula Rio,*** *Ciemat, Madrid ;* ***Luigi Naldini,*** *SR-Tiget, Milan*

11:00 Erik Sontheimer, *UMASS*
INV89: Advanced Precision Genome Editing Platforms

11:30 Anna Ceresetto, *University of Trento*
INV90: From CRISPR to TnpB: Exploring the Landscape of Compact Genome Editors

12:00 Christof Fellmann, *CRISPR-X, CRISPR Therapeutics*
OR096: Single-dose in-vivo gene correction of AATD via LNP-delivered SyNTase editors

12:15 Foteini Christou, *King's College London*
OR097: Enhancement of homology directed repair in post-mitotic cardiomyocytes

12:30 Leah Helton, *Shape Therapeutics*
OR098: Targeted Knockdown of Alpha Synuclein in the Brain Supports the Therapeutic Development of SHP-201 for Parkinson's Disease

12:45 Maria Carmina Castiello, *SR-Tiget, Milan*
OR099: Preclinical advances for the translation of HDR-mediated correction of RAG1 gene in human hematopoietic stem and progenitor cells

11:00-13:00 · PARALLEL · Room Parallel D

SESSION 12d: Hematopoietic & PID II

Chairs: ***Lisa Ott de Bruin,*** *Leiden University Medical Center;* ***Michela Milani****, SR-Tiget, Milan*

11:00 Sandra Le Quellec, *CSL Behring*
INV91: AAV-based gene therapy for haemophilia B: the way to market authorization and future clinical development

11:30 Giuliana Ferrari, *SR Tiget, Milan*
INV92: Gene therapy for beta-thalassemia: towards the new academic trial

12:00 Kerstin Geiger, *Institute for Transfusion Medicine and Gene Therapy and Center for Chronic Immunodeficiency (CCI), Medical Center*
OR103: Base editing restores cellular phenotype of T cells of patients with Hyper-IgE-Syndrome

12:15 Chantal Lagresle-Peyrou, *UMR1163, INSERM, Paris Descartes University Sorbonne Paris Cité, Imagine Institute*
OR100: ex vivo gene therapy for Artemis deficiency: results of the French ARTEGENE phase I/II clinical trial

12:30 Piergiuseppe Quarato, *SR-Tiget, Milan*
OR101: Development of an epigenome editing strategy for the treatment of β-hemoglobinopathies

12:45 Michaela Semeraro, *Hôpital Necker-Enfants Malades, Paris*
OR102: From Cure to Flare: Targeting Post-Gene Therapy Inflammation in Wiskott-Aldrich syndrome

FRIDAY 10 OCTOBER

13:00-14:30 · Pavilion 2
LUNCH

13:30-14:30 · Room Parallel B
EuroGCT Lunchtime Symposium
Policy and regulatory strategies: current and prospective impact on gene and cell therapy development
Chairs: ***Aurélie Mahalatchimy,*** *Aix-Marseille University*

13:30 Anna-Pia Papageorgiou, *DG Research and Innovation, European Commission*
European Union policies and their impact on ATMP Research and Innovation

13:50 Emmanuel Dequier, *French Ministry of Research*
The 2022 French Strategy on biotherapies and bioproduction of advanced therapies: results after 3 years launch and perspectives

14:10 Marcos Timon, *The Spanish Agency of Medicines and Medical Devices (AEMPS)*
Support for ATMPs development in Spain: the central role of the Spanish Medicines Agency (AEMPS)

14:30-16:30 · PLENARY · Auditorium
SESSION 13 Closing: Stem cells: from models to therapies
Chairs: ***Axel Schambach,*** *Hannover Medical School;* ***Ricardo Pardal,*** *University of Seville*

14:30 Elizabeth Ng, *Murdoch Children's Research Institute, Melbourne*
INV93: iHSCs: engrafting haematopoietic stem cells generated from induced pluripotent stem cells.

15:00 Lorenz Studer, *Memorial Sloan Kettering Cancer Center*
INV94: Developing a cell-based therapy for Parkinson's disease

15:30 Isabel Fariñas, *University of Valencia*
INV95: The regulation of adult stem cell quiescence

16:00 Ricardo Pardal, *University of Seville*
INV96: Carotid body cell therapy for Parkinson's disease

16:30-16:45 · PLENARY · Auditorium
Concluding remarks

16:45-18:00 · Registration area
Closing drinks