CONGRESS PROGRAMME

ESGCT 31st Congress in
collaboration with SITGEC
22-25 October 2024 | La Nuvola, Rome

09:00-13:00 · Meeting Room 1 and Meeting Room 2
EDUCATION SESSION

10:00-13:00 · Plenary Hall
CLINICAL TRIALS AND COMMERCIALISATION WORKSHOP

TUESDAY 22 OCTOBER

12:00-14:00 · Registration Foyer
REGISTRATION

14:00-16:30 · Plenary Hall · PLENARY
SESSION 1: ESGCT 2024 Opening: Gene Therapy for Genetic Diseases

Chairs: ***Juan Bueren****, CIEMAT/CIBERER/IIS. F. Jiménez Díaz, Madrid;* ***Alberto Auricchio,*** *Tigem, Naples;* ***Luigi Naldini,*** *SR-Tiget, Milan*

Juan Bueren, *CIEMAT/CIBERER/IIS. F. Jiménez Díaz, Madrid*; **Alberto Auricchio,** *Tigem, Naples*; **Luigi Naldini,** *SR-Tiget, Milan*
Welcome

Alberto Auricchio, *Tigem, Naples*
INV01: ExpEditing AAV gene therapy

Yilai Shu, *Fudan University, Shanghai*
INV02: AAV1-hOTOF Gene Therapy for Autosomal Recessive Deafness 9

Franco Locatelli, *IRCCS, Ospedale Pediatrico Bambino Gesù Rome, Catholic University of the Sacred Heart, Rome*
INV03: Haemoglobinopathies gene therapy

Amit Khera, *Verve Therapeutics, Cambridge, MA*
INV04: Developing single-course gene editing medicines to treat cardiovascular disease

16:30-17:00 · Forum and Registration Foyer
COFFEE BREAK

17:00-19:30 · Plenary Hall · PARALLEL
SESSION 2a: The challenges of preclinical development – how host factors and species-specific differences impact translation

Chairs: ***Hildegard Büning,*** *Hannover Medical School;* ***Stylianos Michalakis****, LMU, Munich*

Luk Vandenberghe, *Harvard Medical School*
INV05: Bridging the species gap in AAV development

Juliette Hordeaux, *University of Pennsylvania*
INV06: Safety profile of recombinant AAV vectors in nonhuman and human primates: bench to bedside and back

Marina Stavrou, *The Cyprus Institute of Neurology and Genetics, Nicosia*
OR001: Dose escalation studies in mice and NHPs confirm biodistribution, target engagement and safety of AAV9-miR871 and support its translation for the treatment of CMT1A neuropathy

Linus Wiora, *German Center for Neurodegenerative Diseases (DZNE), Tübingen*
OR002: Developing a screening platform for AAV tropism in iPSC-derived neural cells

Sumitava Dastidar, *University College London*
OR003: A human, multi-lineage and multi-organoid platform to assess tropism, toxicity and efficacy of neuromuscular gene therapy vectors

Florencia Haase, *Children's Medical Research Institute, Westmead*
OR004: Understanding the involvement of AAVR in AAV entry and transduction of the central nervous system

Randy Chandler, *National Institue of Health, Washington*
OR005: The genomic architecture of 24 carcinogenic recombinant-AAV integrations in mice: Implications for human gene therapy safety

Pedram Moeini Gavgani, *Center for Applied Medical Research (CIMA), University of Navarra, Pamplona*
OR006: Transcriptional findings of unfolded protein response/integrated stress response in the liver of non-human primates subjected to high doses of recombinant AAV vector

TUESDAY 22 OCTOBER

17:00-19:30 · Meeting Room 1 · PARALLEL

SESSION 2b: Lentiviral Vectors / Integrative Vectors

Chairs: ***Axel Schambach,*** *Hannover Medical School;* ***Giuliana Ferrari,*** *SR-Tiget, Milan*

Stefan Radtke, *Fred Hutchinson Cancer Center, Seattle*
INV07: Targeted viral vectors for in vivo gene therapy

Trent Spencer, *Emory University School of Medicine, Atlanta*
INV08: A first in human clinical study using lentiviral vector transduced CD34+ cells encoding a novel FVIII transgene for the treatment of hemophilia A

Eugenio Montini, *SR-Tiget, Milan*
INV87: Monitoring Success: The Long-term Safety and Efficacy of Hematopoietic Stem Cell Gene Therapy

Loukia Touramanidou, *Great Ormond Street Institute of Child Health, University College London*
OR007: In vivo lentiviral gene therapy for distal urea cycle defects.

Alessio Cantore, *SR-Tiget, Milan*
OR008: Comparison of different lentiviral vectors allows the selection of an efficient and safe vector design for in vivo gene therapy of homozygous familial hypercholesterolemia

Steicy Sobrino, *Imagine Institute, Paris*
OR010: Severe inflammation and lineage skewing are associated with poor engraftment of engineered hematopoietic stem cells in patients with sickle cell disease

Michael Rothe, *Hannover Medical School*
OR011: Predicting the risk of insertional mutagenesis with SAGA-Q

Cristina Colleoni, *SR-Tiget, Milan*
OR012: A mouse model of genotoxicity in HSPC gene therapy to investigate the persistence of senescent cells

17:00-19:30 · Meeting Room 2 · PARALLEL

SESSION 2c: Gene therapy of sensory diseases

Chairs: **Ivana Trapani**, *Tigem Naples,* **Robin Ali,** *King's College London*

Robin Ali, *King's College, London*
INV09: Gene Therapy in children with severe retinal dystrophy associated with AIPL1 gene defects

Elvir Becirovic, *University of Zurich*
INV10: mRNA trans-splicing dual AAV vectors for treatment of Stargardt disease and Usher syndrome type 1B

Alvin Luk, *HuidaGene Therapeutics*
OR013: Results from LIGHT, a first-in-human AAV9-gene replacement therapy trial of HG004, in children and adults with RPE65-associated Leber's congenital amaurosis

Arjun Padmanabhan, *Tigem, Naples*
OR014: AAV HITI for therapy of dominant Retinitis Pigmentosa.

Brendan Lilley, *RegenX Bio*
OR015: Suprachoroidal delivery of a vectorized complement inhibitor using a novel AAV capsid as a potential treatment for dry age-related macular degeneration

Manohar Bance, *Cambridge University*
OR016: Preliminary safety and efficacy of DB-OTO gene therapy in pediatric patients with profound deafness due to otoferlin variants: The CHORD phase 1/2 open-label trial

Rita Ferla, *AAVantgarde BIO s.r.l.*
OR017: Safety and expression of intein-based Dual AAV8.ABCA4 in the non-human primate retina

Laure Blouin, *SparingVision, Paris*
OR018: PRODYGY: A first-in-human trial of rod-derived cone viability factor (RdCVF) gene therapy in subjects with rod-cone dystrophy

TUESDAY 22 OCTOBER

17:00-19:30 · Auditorium · PARALLEL

SESSION 2d: Immunotherapy and CAR T-Cells I

Chairs: **Waseem Qasim,** *UCL Institute of Child Health, London;* **Chiara Magnani,** *University Hospital Zürich and University of Zürich*

Eliana Ruggiero, *Universita Via-Salute San Raffaele, Milan*
INV11: TCR-engineered T cell therapy comes of age: generating safe and effective treatments for acute myeloid leukemia

William Nyberg, *Karolinska Institute*
INV12: In vivo site-specific engineering to reprogram T cells

Tali Stauber, *Tel Aviv University*
OR019: CAR-T cells targeting the human IgE-B-cell-receptor specifically abolish IgE production by human B cells and eliminate IgE+ cells in vivo

Martina Spiga, *SR-Tiget, Milan*
OR020: Cytosine base editor ameliorates the safety profile of TCR edited T cells for the Adoptive Cell Therapy of gastrointestinal tumors

Anders Laustsen, *UNIKUM Therapeutics*
OR021: Design of Programmable Immune Reactive Cells (PIRCs) with precision type I Interferon and cytotoxicity response in solid tumors

Samik Basu, *Cabaletta Bio*
OR022: Correlative findings following DSG3-CAART infusion with and without preconditioning in patients with Pemphigus Vulgaris (DesCAARTesTM trial)

Dorothee Haas, *University Hospital of Würzburg*
OR023: Restoration of the autophagy machinery promotes long-term persistence and efficacy of CAR-T cells and adoptive cellular products

Roland Preece, *University College London*
OR024: Base-edited CAR38 T cells evade fratricide and immune rejection while delivering potent anti-leukemic affects:

19:30-21:00 · Forum

WELCOME RECEPTION and POSTER SESSION I (Uneven numbers up to P0519)

WEDNESDAY 23 OCTOBER

08:00-08:30 · Registration Foyer
REGISTRATION

08:30-10:30 · Plenary Hall · PARALLEL
SESSION 3a: AAV vectors as tools in gene therapy of rare diseases – recent development to improve efficacy and safety
Chairs: **Juliette Hordeaux**, *University of Pennsylvania,* **Fatima Bosch**, *UAB, Barcelona*

Gloria Gonzalez-Aseguinolaza; *CIMA, University of Navarra, Pamplona*
INV13: Gene therapy for liver diseases — progress and challenges

Ian Alexander, *University of Sydney*
INV14: AAV vectors and the paediatric liver as a therapeutic target

Guo-jie Ye, *Exegenesis Bio*
OR025: Efficient Retinal Transduction and Excellent Inhibitory Effect on Retinal Detachment of EXG202 delivered by intravitreal injection in NHP and a Severe Neovascularization and vascular leakage Mouse Model

Shanzhong Zhang, *Otovia Therapeutics*
OR026: OTOV101 Gene therapy for autosomal recessive deafness 9: a multicenter, open-label, single-arm, investigator initiated intervention study

Serge Braun, *Genethon, Evry*
OR027: GNT0004, Genethon's AAV8 vector-delivered microdystrophin gene therapy for Duchenne muscular dystrophy: first data from Phase 1/2 part of GNT-016-MDYF all-in-one clinical trial in ambulant boys

Francesca Ferrante, *Spur Therapeutics*
OR028: Results from GALILEO-1, a first-in-human clinical trial of FLT201 gene therapy in patients with Gaucher disease Type 1

08:30-10:30 · Meeting Room 1 · PARALLEL
SESSION 3b: Oncolytic Therapy and Cancer Gene Therapy
In collaboration with the JSGCT
Chairs: **Vincenzo Cerullo,** *University of Helsinki,* **Masahiro Toda,** *Keio University School of Medicine, Tokyo*

Elisa Scarselli, *Nouscom, Rome*
INV15: Genetic vaccines journey: from cancer treatment to interception

Masahiro Toda, *Keio University School of Medicine, Tokyo*
INV16: Gene therapy for brain tumors using neural stem cells derived from genome-edited iPS cells

Claudia Pinacchio, *Bambino Gesù Children's Hospital, Rome*
OR029: Enhanced Immunotherapy for High-Grade Gliomas Using Oncolytic Virus Armed with Co-Stimulatory Molecule and EphA2-Engager

Fritiof Åkerström, *Asgard Therapeutics AB, Medicon Village, Lund*
OR030: A cancer immunotherapy modality based on dendritic cell reprogramming *in vivo*

Giovanna Giacca, *SR-Tiget, Milan*
OR031: An hemato-chimeric mouse model hosting human liver-resident macrophages for translational studies using in vivo lentiviral-based gene therapies

Jella van de Laak, *Faculty of Health, Medicine & Life Sciences (FHML), Maastricht University*
OR032: Genetically modified bacteria as anti-cancer Trojan horse; intratumoural delivery of immunotherapy by Clostridium sporogenes

08:30-10:30 · Meeting Room 2 · PARALLEL
SESSION 3c: Infectious Diseases / Vaccines
Chairs: ***Karine Breckpot,*** *VUB, Brussels;* ***Chantal Pichon,*** *Centre de Biophysique Moléculaire, CNRS, Orléans*

Patrick Arbuthnot, *University of Witwatersrand*
INV17: Formulation of mRNA sequences in bio-renewable lipid nanoparticles to counter infection with *Mycobacterium tuberculosis* and other pathogens

Asaf Poran, *BioNTech*
INV18: A multivalent mRNA monkeypox virus vaccine (BNT166) protects mice and macaques from orthopoxvirus disease

Kristie Bloom, *University of the Witwatersrand*
OR033: Mycobacterium Tuberculosis polyprotein mRNA vaccines designed to augment protein sorting enhance immunogenicity and protection in preclinical studies

Anja Ehrhardt, *Witten/Herdecke University*
OR034: Novel adenovirus vaccine vectors lacking binding to the thrombosis associated Platelet Factor 4 protein

Norbert Pardi, *University of Pennsylvania*
OR035: Tailoring the adjuvanticity of lipid nanoparticles by PEG lipid ratio and phospholipid modifications

Angelo Raggioli, *Reithera Srl*
OR036: GRAd as vaccine platform for COVID-19, HIV, and global health

WEDNESDAY 23 OCTOBER

08:30-10:30 · Auditorium · PARALLEL

SESSION 3d: Metabolic Diseases: Preclinical

Chairs: **Yoshikatsu Eto,** *Tokyo Jikei University;* **Nicola Brunetti-Pierri,** *TIGEM, Naples*

Dwight Koeberl, *Duke University, Durham, NC*
INV19: Gene Editing for Glycogen Storage Disease

Brian Bigger, *The University of Edinburgh*
INV20: Stem cell gene therapy for Childhood Dementias – a Sanfilippo story

Louisa Jauze, *Genethon, Evry*
OR037: Liver-directed AAV gene transfer corrects hypoglycemia and metabolic impairment in a GSDIII mouse model in the long term.

Federica Esposito, *Tigem, Naples*
OR038: Safe and effective liver-directed AAV-mediated homology-independent targeted integration in mouse models of inherited diseases

Stefania Crippa, *SR-Tiget, Milan*
OR039: Pre-clinical development of an ex-vivo lentiviral-based Hematopoietic Stem/Progenitor Cells-Gene Therapy (HSPC-GT) for Mucopolysaccharidosis type IVA (MPSIVA) as part of an innovative GT platform approach for LSDs with skeletal involvement.type II

Fabio Catalano, *Erasmus MC University Medical Center, Rotterdam*
OR040: Domain-substituted IGF2 tag to modulate receptor targeting during lentiviral gene therapy for Mucopolysaccharidosis type II

10:30-11:00 · Forum and Registration Foyer

COFFEE BREAK

11:00-13:00 · Plenary Hall · PLENARY

SESSION 4: Cancer Gene Therapy

Chairs: **Chiara Bonini**, *San Raffaele Scientific Institute, Milan,* **Waseem Qasim,** *UCL London*

Gianpietro Dotti, *UNC School of Medicine, Chapel Hill, NC*
INV21: CAR cell therapies: new opportunities

Concetta Quintarelli, *Bambino Gesù Children's Hospital, IRCCS, Rome*
INV22: CAR T Cells for Treating Pediatric Cancers: Challenges and Opportunities

Bernhard Gentner, *Ludwig Institute for Cancer Research, Lausanne*
INV23: Genetic reprogramming of tumor-associated myeloid cells to awaken anti-cancer immunity

Marta Alonso, *University of Navarra, Pamplona*
INV24: Overcoming oncolytic virus resistance to improve the treatment of pediatric brain tumors

13:00-14:00 · Forum and Registration Foyer

LUNCH

13:30-15:00 · Concourse Level -1 and Mezzanine Concourse

POSTER SESSION II (Even numbers up to P0520)

13:30-14:30 · Meeting Room 1

LUNCHTIME SYMPOSIUM: PlasmidFactory - A spotlight on plasmid and minicircle DNA starting materials for AAV vectors

Chairs: **Eduard Ayuso,** *DINAMIQS*; **Dr. Marco Schmeer,** *PlasmidFactory GmbH*

Hildegard Büning, *Hannover Medical School (MHH)*
Improving the quality of AAV vector preparations by exchanging plasmids by minicircles

Michela Gentile, *ReiThera srl*
Optimization of the two-plasmid system for AAV production

Kristian Müller, *Bielefeld University*
A hitchhiker's guide to ITR stability

Ram Shankar, *PlasmidFactory GmbH*
ITR maintenance and in-depth analysis for AAV transfer plasmids

13:30-14:30 · Auditorium

LUNCHTIME SYMPOSIUM: Sartorius - Setting the Standard in Differentiated Development: Innovative Approaches to Scalable PSC Production

Chair: **Lorraine Borland,** *Sartorius*

Lorraine Borland, *Sartorius*
Introduction

Victor Buñuel Sorribas, *Novo Nordisk*
Scalable Manufacturing of Pluripotent Stem Cell Therapies: Revolutionizing Cell Harvesting with Ksep Systems

Robert Zweigerdt, *Hannover Medical School*
Advanced manufacturing of hPSC-cardiomyocytes and preclinical transplantation for heart repair

Mark A. Skylar-Scott, *Betty Irene Moore Children's Heart Center*
Liter Scale Production of iPSC-Derived Cardiomyocytes

WEDNESDAY 23 OCTOBER

13:30-14:15 · Satellite Room - M1

LUNCHTIME WORKSHOP: How to get published

Kevin Davies, *Mary Ann Liebert*
Thomas Gallagher, *University of Massachusetts*

Kevin Davies, the founding editor of *Nature Genetics* and Executive Editor of *The CRISPR Journal*, and Thomas Gallagher, the Managing Editor of *Human Gene Therapy*, will discuss the publishing process to provide insights from the perspective of a journal and publishing company to help scientists navigate the manuscript submission, peer review, and publication process. More information >

Attendees must sign up in advance. Register at https://forms.office.com/e/YnrRv0EM3Y

15:00-17:00 · Plenary Hall · PLENARY

SESSION 5: Advanced Technologies for the Treatment of Human Diseases

Chairs: **Toni Cathomen,** *University of Freiburg*, **Matthew Porteus,** *Stanford University*

Angelo Lombardo, *SR-Tiget, Milan*
INV25: Programming permanent gene repression by epigenetic editing

Zoya Ignatova, *University of Hamburg*
INV26: Unlocking the unreachable: harnessing tRNA therapeutics for rare genetic conditions

Paula Rio, *Ciemat/CIBERER/IIS-Fundación Jiménez Díaz, Madrid*
INV27: Hematopoietic stem cell gene therapy: Overcoming challenges in Fanconi anemia

Samuel Sternberg, *Columbia University, New York, NY*
INV28: Discovery and development of CRISPR-associated transposases for RNA-guided gene insertion

17:00-17:30 · Forum and Registration Foyer

COFFEE BREAK

17:30-19:30 · Plenary Hall · PARALLEL

SESSION 6a: Beyond conventional: next generation vectors in gene and cell therapy: Part 1 (Capsid)

Chairs: ***Luk VandenBerghe,*** *Harvard Medical School;* ***Gloria Gonzalez Aseguinolaza,*** *CIMA, University of Navarra, Pamplona*

Hildegard Büning, *Hannover Medical School*
INV29: Capsid engineering to empower the adeno-associated virus (AAV) vector system for its new "fields of action"

Fabian John, *Paul-Ehrlich-Institut, Langen*
OR041: Selective transduction of lymphoid cell subsets with bispecific DART-AAVs

Angela Enrica Araujo, *Hannover Medical School*
OR042: Development of engineered AAV variants to target T lymphocytes in vivo

Alaa Siam, University College London
OR043: Investigating the safety, efficacy and dynamics of lentiviral and AAV gene therapies in a mouse model of maple syrup urine disease

Qizhao Wang, *AAVnerGene Inc*
OR044: AAV(BBB)s: Novel AAV Variants with 500-fold Higher BBB Crossing Efficiency in NHP

Andrea Pérez Iturralde, *The University of Sydney, Westmead*
OR045: Towards the translation of an AAV-mediated gene therapy for an incurable disease

Yumi Sano, *Heidelberg University*
OR046: Critical role of VP3 N-terminal residues and variable capsid regions for bocaparvovirus transduction

17:30-19:30 · Meeting Room 1 · PARALLEL

SESSION 6b: Skin, Pulmonary and Skeletal Diseases

Chairs: **Alain Hovnanian,** *Institut Imagine, Paris;* **Uta Griesenbach,** *Imperial College, London*

Marta Garcia Diez, *CIEMAT/CIBERER, Madrid*
INV30: Personalized preclinical gene editing protocols for ex vivo and in vivo correction of rare skin disease

Sarah Hedtrich, *Charité, Berlin*
INV31: In situ gene editing as a topically applicable treatment option for genodermatoses

Uta Griesenbach, *Imperial College, London*
INV32: Gene Therapy for Rare Lung Diseases

Alex Bassons Bascuñana, *Ciemat, Madrid*
OR047: Efficient base and prime editing to correct COL7A1 patogenic variants in primary RDEB patient keratinocytes

Simone Ponta, *ETH Zurich*
OR048: Biofabrication of a hyaline cartilage graft mimicking native architecture from genetically engineered MSCs with enhanced chondrogenic potential

WEDNESDAY 23 OCTOBER

17:30-19:30 · Meeting Room 2 · PARALLEL

SESSION 6c: Non Viral Vectors / Nanotechnology I

Chairs: ***Zoltan Ivics,*** *Fraunhofer Institute for Cell Therapy and Immunology, Leipzig;* ***Ray Schiffelers,*** *University Medical Center Utrecht*

Frank Buchholz, *Technical University Dresden*
INV33: Engineering Designer-Recombinases for therapeutic genome editing

Ross Wilson, *University of Berkeley*
INV34: Highly efficient in vivo editing of neurons via peptide-mediated delivery of CRISPR enzymes

Russell Monds, *Generation Bio, Cambridge MA*
OR049: iqDNA is an engineered DNA cargo that avoids innate immune activation while retaining durable transgene expression

Jacek Lubelski, *NanoCell Therapeutics*
OR050: tLNPs can effectively deliver DNA to T-cells and generate long-acting CAR-T cells in vivo.

Guillaume Mondon, *Paris Cité University*
OR051: Exon skipping using palmitoyl conjugated tricyclo-DNA antisense oligonucleotides restores protein expression in vivo in recessive dystrophic epidermolysis bullosa.

Thomas Hansen, *Circio*
OR052: Optimization of in vitro and in vivo performance of circVec, a vector-based circular RNA expression platform for enhanced gene therapy

17:30-19:30 · Auditorium · PARALLEL

SESSION 6d: Hematopoietic, PID and Bleeding I

Chairs: ***Claire Booth,*** *University College London;* ***Jose Carlos Segovia Sanz,*** *Ciemat/Ciberer, Madrid*

Amit Nathwani, *University College London*
INV35: Gene Therapy for Hemophilia: From bench side to Market Authorization

Don Kohn, *UCLA*
INV36: Hematopoietic Stem Cell Gene Therapy

Emmanuelle Six, *Imagine Institute Paris*
OR053: Second occurrence of LMO2-associated clonal T cell proliferation 20 years after gamma-retrovirus-mediated gene therapy for SCIDX1

Martina Fiumara, *SR-Tiget, Milan*
OR054: Modeling of VEXAS syndrome by Base Editing uncovers poisoning of healthy hematopoiesis as an unanticipated mechanism driving clonal dominance during aging

Michela Milani, *SR-Tiget, Milan*
OR055: In vivo gene transfer into haematopoietic stem and progenitor cells for the treatment of Fanconi Anemia

Frank Staal, *Leiden University Medical Center*
OR056: Gene therapy for Recombinase deficient-SCID and hypomorphic RAG disease

THURSDAY 24 OCTOBER

08:30-09:00 · Registration Foyer
REGISTRATION

09:00-11:00 · Plenary Hall · PARALLEL
SESSION 7a: Improving Gene Editing Outcome
Chairs: ***Paula Rio****, Ciemat/Ciberer, Madrid;* ***Julian Grünewald,*** *TUM Munich*

Raffaella Di Micco, *SR-Tiget, Milan*
INV37: Mechanistic insights to advance HSC-based gene editing applications

Matthew Porteus, *Stanford University*
INV38: Improving Homology Directed Repair Genome Editing

Lei Lei, *University of Freiburg*
OR057: Cell-Type and Time-Resolved Genotoxicity of Base Editing

Daniele Canarutto, *SR-Tiget, Milan*
OR058: Efficient long range gene editing and enrichment of lymphocytes and hematopoietic stem and progenitor cells with the desired editing outcome

Giandomenico Turchiano, *University College London*
OR059: Chromosomal aberrations, NHEJ scarless repair, recurrent nuclease cleavage and DSB half-life; all at once.

Deborah Cipria, *SR-Tiget, Milan*
OR060: Towards safer engineering of T cells for cancer immunotherapy by polyfunctional editing

09:00-11:00 · Meeting Room 1 · PARALLEL
SESSION 7b: Hematopoietic, PID and Bleeding II
Chairs: ***Amit Nathwani,*** *UCL London,* ***Don Kohn,*** *UCLA*

Denise Sabatino, *University of Pennsylvania*, *Philadelphia, PA*
INV39: Innovations in factor VIII biology for next generation hemophilia A gene therapy

Rosa Bacchetta, *Stanford University School of Medicine, CA*
INV40: IPEX gene therapy trial with the engineered Treg-like cells

Cesare Canepari, *SR-Tiget, Milan*
OR061: Enhancing the potency of in vivo lentiviral gene therapy to hepatocytes for hemophilia

Ivan Krivega, *SonoThera*
OR062: Development of a non-viral genetic medicine for Hemophilia A by targeted transcutaneous ultrasound-mediated gene delivery

Wolfgang Miesbach, *Goethe University Hospital, Frankfurt am Main*
OR063: Stable factor IX expression and sustained reductions in factor IX use 7 years after gene therapy with AMT-060 in adults with haemophilia B

Pervinder Sagoo, *Orchard Therapeutics, London*
OR064: Haematopoietic stem cell gene therapy as a treatment for NOD2-deficient severe Crohn's Disease

09:00-11:00 · Meeting Room 2 · PARALLEL
SESSION 7c: Cardio and Muscular
Chairs: ***Mauro Giacca,*** *King's College London;* ***Eduard Ayuso,*** *DINAMIQS*

Matthew Wood, *Oxford University*
INV41: Accelerating genome based therapies for rare neuromuscular diseases

Dan Peer, *University of Tel Aviv*
INV42: Cell Specific Delivery of RNAs Using Targeted Lipid Nanoparticles: from Vaccines to Therapeutic Genome Editing.

Mateusz Tomczyk, *King's College London*
OR065: mRNA therapy for acute myocardial infarction

Edith Renaud, *Genethon Evry*
OR066: Ten-year efficacy of gene therapy in a canine model of X-linked myotubular myopathy

Yann Chong Tan, *Nuevocor Pte. Ltd.*
OR067: NVC-001 – an AAV gene therapy for LMNA-related dilated cardiomyopathy

Laura Lalaguna, *CNIC, Madrid*
OR068: AAV delivery of wild type TMEM43 as a novel therapeutic approach for arrhythmogenic right ventricular cardiomyopathy type 5

THURSDAY 24 OCTOBER

09:00-11:00 · Auditorium · PARALLEL

SESSION 7d: CNS gene therapy

Chairs: ***Nathalie Cartier****, AskBio, Paris;* ***Alberto Auricchio,*** *Tigem Naples*

Krystof Bankiewicz, *AskBio*
INV43: Clinical Progress and Plans for Aav2GDNF Gene Therapy for Parkinson's Disease

Isabel Aznarez, *Stoke Therapeutics*
INV44: The zorevunersen story: insights from the development of the first potential disease-modifying medicine for Dravet syndrome

Florian Eichler, *Massachusetts General Hospital*
OR069: Results from the CANaspire Gene Therapy Trial for Canavan Disease: Safety, Biomarker, Imaging, and Clinical Outcome Data from the Completed Low-dose Cohort

Sue Browne, *Passage Bio, Philadelphia PA*
OR070: Non-clinical and early clinical development of PBFT02, an AAV gene therapy for FTD with GRN mutations (FTD-GRN)

Dolan Sondhi, *Weill Cornell Medical College*
OR071: Twenty-year Survival Analysis of CNS AAV2-mediated Gene Therapy for CLN2 Disease

Anne Hillen, *SR-Tiget, Milan*
OR072: Targeting astrocytes with editing technologies to treat Alexander Disease

09:00-10:30 · Satellite Room -M1 / PARALLEL

SESSION 7e: T2EVOLVE/JOIN4ATMP Roundtable Discussion: Accelerating Cell and Gene Therapy development through Extrapolation and Platform Approaches

Chairs: ***Carmen Sanges,*** *University Hospital Würzburg;* ***Delphine Ammar,*** *Astellas (IMI T2Evolve)*

Carmen Sanges, *University Hospital Würzburg* and **Delphine Ammar,** *Astellas (IMI T2Evolve)*
Introduction

Mark Stewart, *Friends of Cancer Research*
Extrapolation framework for accelerating next generation therapies

Macarena Roman Alonso, *Vall D'Hebron Institute of Oncology (VHIO)*
Case Study: A parent-child platform approach for the development of CAR T cell therapies targeting solid tumors using a common retroviral vector and manufacturing process

Maria Ester Bernardo, *Telethon / Join4ATMP*
Case Study: An innovative platform approach for the development of ex-vivo gene therapies (HSC-GT) for lysosomal storage disorders (LSD) with skeletal involvement

Roundtable Discussion
Academic developer: Julio Delgado, *Hospital Clinic Barcelona*
Industry developer: Agnes Yeboah, *Bristol Myers Squibb*
Industry developer: William McAree, *Kite Gilead*
EMA representatives: Illona Reischl, *AGES & EMA CAT Chair &* **Paolo Foggi,** *AIFA*

11:00-11:30 · Forum and Registration Foyer

COFFEE BREAK

11:30-13:30 · Plenary Hall · PLENARY

SESSION 8: Presidential Symposium

Chairs: **Juan Bueren***, CIEMAT/CIBERER/IIS. F. Jiménez Díaz, Madrid;* **Luigi Naldini***, SR-Tiget, Milan,* **Alberto Auricchio***, Tigem, Naples*

Juan Bueren, *Ciemat, Madrid*
INV45: Presidential address

Douglas Higgs, *University of Oxford*
INV46: Switching on an embryonic gene to cure an adult disease

OUTSTANDING ACHIEVEMENT AWARD
Chiara Bonini, *San Raffaele Hospital Milan*
INV47: T-cell engineering to fight cancer and autoimmunity

CAREER PROGRESSION AWARD
Manlio Fusciello, *University of Helsinki*
OR073: Cancer Vaccines: Anti-tumoral T cell therapy on demand

CAREER PROGRESSION AWARD
Serena Scala, *SR-Tiget, Milan*
OR074: Studying human Hematopoietic Stem/Progenitor Cell trafficking: from basic biology to clinical translation

Award ceremony for early careers and travel awards

THURSDAY 24 OCTOBER

13:30-14:30 · Forum and Registration Foyer
LUNCH

13:30-14:00 · Plenary Hall
ESGCT AGM

14:00-15:00 · Meeting Room 1
LUNCHTIME SYMPOSIUM: MaxCyte - Combining CRISPR and Transposon-based Technologies for Improved CAR-T Therapies

Juan Roberto Rodriguez-Madoz, *Cima Universidad de Navarra*

14:00-15:00 · Auditorium
LUNCHTIME SYMPOSIUM: ERC - Unlocking success: insights from an ERC Project Adviser and ERC Grantees to maximize your chances

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

Paola Cattaneo, *National Research Council (IRGB-CNR), Milan*
Marc Güell, *Pompeu Fabra University, Barcelona*

Roundtable

14:00-15:30 · Concourse Level -1 and Mezzanine Concourse
POSTER SESSION III (Uneven numbers from P0521 to P1045)

15:30-17:30 · Plenary Hall · PARALLEL
SESSION 9a: *in vivo* Gene Editing in Preclinical Models

Chairs: **Annarita Miccio,** *Institut Imagine Paris*,

Gerald Schwank, *University of Zurich*
INV48: In vivo genome editing in mice and macaques using mRNA-LNP delivery

Cynthia Dunbar, *NHLBI, NIH*
INV49: Impact of Gene Editing on HSPC Dynamics in Macaques

Gabriele Casirati, *Boston Children Hospital*
OR075: Epitope editing combined with extended schedule anti-KIT antibody treatment enhances immune-based in vivo selection of multiplex genome-engineered cells

Michael Holmes, *Tessera Therapeutics*
OR076: RNA Gene Writers drive therapeutically relevant in vivo correction of monogenic disease mutations in the liver and hematopoietic stem cells

Jeremy Duffield, *Prime Medicine*
OR077: LNP delivered Prime Editors restore glycemic control in humanized rodent models of Glycogen Storage Disease Type 1b (GSD1b)

Maëlle Ralu, *Genethon Evry*
OR078: CRISPR-Cas9 mediated endogenous utrophin upregulation improves Duchenne Muscular Dystrophy

15:30-17:30 · Meeting Room 1 · PARALLEL
SESSION 9b: Immune Responses to Gene Therapy

Chairs: ***Carmen Unzu,*** *CIMA Universidad de Navarra, Pamplona;* ***Federico Mingozzi,*** *Nava Therapeutics*

Anna Kajaste-Rudnitski, *SR-Tiget, Milan / University of Pavia*
INV50: Dissecting Cell Intrinsic Innate Immunity against Viral Vectors

Sophie Janssens, *University of Ghent and VUB, Belgium*
INV51: Lipid nanoparticles induce homeostatic dendritic cell maturation and do not contain intrinsic adjuvant activity.

Fraser Wright, *Stanford University*
OR079: Engineering albumin-binding domains into the capsid protein of AAV: A transient cloaking mechanism to block antibody binding for improved vector safety and efficacy

Andrea Annoni, *SR-Tiget, Milan*
OR080: Co-stimulatory blockade regimen prevents anti-transgene and anti-vector immune responses in hemophilia A mice after *in vivo* LV gene therapy.

Allwyn Pereira, *University of Nantes*
OR081: Assessing molecular mechanisms of microglial mediated inflammation in retinal gene therapy

Svetlana Atasheva, *Emory University*
OR082: Immunological Safety of Adenoviral Vector Gene Therapy.

THURSDAY 24 OCTOBER

15:30-17:30 · Meeting Room 2 · PARALLEL

SESSION 9c: Immunotherapy and CAR-T Cells II

Chairs: ***Monica Casucci,*** *IRCCS San Raffaele Scientific Institute, Milan;* ***Carmen Sanges,*** *University of Würzburg*

Chiara Magnani, *University Hospital Zürich and University of Zürich*
INV52: Activity, biological properties, and safety of non-viral CAR T cells engineered with Sleeping Beauty for hematological malignancies

Sara Ghorashian, *University College London*
INV53: Overcoming current limitations in CAR T cell therapy of ALL

Arianna Pocaterra, *IRCCS San Raffaele Scientific Institute, Milan*
OR083: T cell therapy for PDAC: overcoming resistance by tumor/stroma dual-targeting CAR-T cells

Rupert Kenefeck, *Quell Therapeutics*
OR084: CAR-Treg cell therapy to induce tolerance in liver transplantation – LIBERATE clinical trial

Mariana Silva, *Vor Bio, Cambridge*
OR085: Novel CD33/CLL-1-directed dual CAR-T cells mediate potent antigen-specific cytolytic activity in mouse models of Acute Myeloid Leukemia (AML)

Paula Rodriguez-Marquez, *Cima Universidad de Navarra. IdiSNA. Pamplona*
OR086: Molecular mechanisms promoting long-term cytopenia after BCMA CAR-T therapy in Multiple Myeloma

15:30-17:30 · Auditorium · PARALLEL

SESSION 9d: Accessibility of Gene Therapy

Chairs: ***Hildegard Büning,*** *Hannover Medical School,* ***Stefano Benvenuti,*** *Fondazione Telethon*

Amit Nathwani, *LifeArc*
INV54:

Sol Ruiz, *AEMPS*
INV55: Regulatory progress

Terry Pirovolakis, *CureSPG50*
INV56: Journey to cure

David Epstein, *University of Granada*
INV57: The economics of gene therapies

Arjan Lankester, *LUMC, Leiden*
INV59: Access to gene therapy, the AGORA perspective

Annette Künkele-Langer, *Join4ATMP*
INV60: Map, join and drive European activities for ATMP development and implementation - JOIN4ATMP

Panel Discussion

17:30-18:00 · Forum and Registration Foyer

COFFEE BREAK

17:30-19:30 · Auditorium · PARALLEL

SESSION 10: Towards the Accessibility of ATMPs for Rare and Ultra-rare Diseases with No Commercial Interest: Is there a role for Hospital Exemption?

Chairs: **Juan Bueren,** *CIEMAT/CIBERER/IIS. F. Jiménez Díaz, Madrid;* **Alessandro Aiuti,** *SR-Tiget, Milan;* **Claire Booth**, *University College London*

Alessandro Aiuti, *SR-Tiget, Milan*
INV61: Challenges in ATMPs development and access to patients for rare and ultra-rare diseases

Paschalia Koufokotsiou, *European commission*
INV62: Current meaning and legislation of Hospital Exemption in the EU

Sol Ruiz, *AEMPS, Madrid*
INV63: Key standards for the harmonization of HE across the EU

Panel Discussion:
Elisabetta Zanon: Director of EU Public Affairs & Advocacy. Alliance for Regenerative Medicine
Cristina Avendaño-Sola: Spanish Network on Advanced Therapies (TERAV)
Johan Prevot: International Patient Organization for Primaries Immunodeficiencies; IPOPI

18:00-19:30 · Concourse Level -1 and Mezzanine Concourse

POSTER SESSION IV (Even numbers from P0522 to P1046)

20:00 · Palazzo Brancaccio

MOLECULAR MINGLE

FRIDAY 25 OCTOBER

08:30-09:00 · Registration Foyer
REGISTRATION

09:00-10:30 · Plenary Hall · PARALLEL
SESSION 11a: Immunotherapy and CAR T cells III
In collaboration with ASGCT

Chairs: ***Paula Cannon,*** *University of Southern California;* ***Chiara Bonini,*** *San Raffaele Hospital, Milan*

Paula Cannon, *University of Southern California*
INV64: Engineering B cells for custom antibody production

David Klatzmann, *UPMC, Sorbonne University*
INV65: Self-sufficiency for an IL-2 partial agonist dramatically improves survival and efficacy of Treg cell therapy

Nils Wellhausen, *University of Pennsylvania*
OR087: Elimination of cellular HIV reservoirs by CCR5/CD45 multiplex base edited CD45 CAR-T cell therapy

Martina Pigazzi, *University-Hospital of Padova, Padua*
OR088: Successful Preclinical Proof-of-Concept Study of a CAR-T Cell Approach Targeting CD84 to Treat Acute Myeloid Leukemia

09:00-10:30 · Meeting Room 1 · PARALLEL
SESSION 11b: Beyond conventional: next generation vectors in gene and cell therapy, part II (AAV genome, genome editing)

Chairs: ***Leszek Lisowski****, Children Medical Research Institute, Sydney;* ***Giuseppe Ronzitti****, Genethon, Evry*

Arun Srivastava, *University of Florida*
INV68: Beyond capsid-modifications: Development of genome-modified AAV vectors

Sibtain Haider, *Universitäts klinikum Freiburg*
OR089: Peptide-assisted tethering of DNA repair effectors to Cas9 for precise genome editing

Caner Günaydin, *Weill Cornell Medical College*
OR090: Genome Editing of Human APOE4 to APOE3 in the Brain of APOE4 Mice

Federica Fioretto, *Tigem, Naples*
OR091: Mutation-independent genome editing approaches for treatment of Stargardt disease

Phillip Tai, *UMass Chan Medical School*
OR092: Novel inverted terminal repeat sequences and flanking proximal regions from serotypes AAV8 and AAV.rh39 show robust promoter-like activities that enhance transgene expression in a tissue-dependent manner

09:00-10:30 · Meeting Room 2 · PARALLEL
PARALLEL 11c: Disease Models / IPS cells / Organoids Cell Therapy

Chairs: ***Markus Grompe,*** *Oregon Health & Science University, Portland;* ***Vania Broccoli,*** *San Raffaele Scientific Institute, Milan / CNR Institute of Neuroscience, Milan*

Benedetta Artegiani, *Princess Máxima Center for Pediatric Oncology, Utrecht*
INV69: Human organoid models for disease modeling

Vania Broccoli, *San Raffaele Scientific Institute, Milan / CNR Institute of Neuroscience, Milan*
INV70: Generation of fully human pluripotent stem cell-derived blood-brain barrier organoids for disease modeling and validation of neurotropic viruses

Maria del Carmen Ortuño Costela, *Berlin Institute of Health*
OR093: Generation of hepatocyte organoids from primary hepatocytes

Martina Nubiè, *Institute for Regenerative Medicine (IREM), University of Zurich*
OR094: Investigating the effects of progranulin reconstitution driven by microglia-directed gene therapy in iPSC-derived neural networks

09:00-10:30 · Auditorium · PARALLEL
SESSION 11d: New Tools and Delivery for Gene Editing

Chairs: ***Angelo Lombardo,*** *SR-Tiget, Milan;* ***Silvia Roman,*** *University of Freiburg*

Daniel Anderson, *Massachusetts Institute of Technology*
INV71: Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing

Jin Soo Kim, *Seoul National University*
INV72: Mitochondrial DNA editing in vitro and in vivo

Laura Torella, *Center for Applied Medical Research (CIMA), University of Navarra, Pamplona*
OR095: Investigating the interplay between DNA repair pathways and recombinant AAV integration into CRISPR-Cas-induced double-strand breaks in vivo

Piergiuseppe Quarato, *SR-Tiget, Milan*
OR096: Development of an epigenome editing strategy for the treatment of β-hemoglobinopathies

FRIDAY 25 OCTOBER

10:30-11:00 · Forum and Registration Foyer
COFFEE BREAK

11:00-13:00 · Plenary Hall · PARALLEL
SESSION 12a: Manufacturing
Chairs: ***Cesar Trigueros,*** *Viralgen, San Sebastian;* ***Mercedes Segura,*** *Elevate Bio*

Nina Kotsopoulou, *AAVantgard bio*
INV73: Production of dual AAV vectors for the delivery of large genes

Marianna Sabatino, *Neogene*
INV74: Non viral based genome engineering for generation of TCR T cell therapy products

Eduard Ayuso, *DINAMIQS*
INV75: Manufacturing AAV vectors: every step matters

Simona Porcellini, *SR-Tiget, Milan*
OR097: Gene correction of HIGM1 CD4+ T cells: a comprehensive analysis of GMP-compliant process performance and product critical quality attributes

Kumar Dhanasekharan, *Voyager Therapeutics*
OR098: Scaling up manufacturing to 1000L and beyond with novel AAV capsids and payloads: From early developability assessment to process development and scale-up.

11:00-13:00 · Meeting Room 1 · PARALLEL
SESSION 12b: Metabolic Diseases: Clinical
Chairs: ***Maria Ester Bernardo,*** *SR-Tiget, Milan,* **Alessia Cavazza**, *University College London*

Julien Baruteau, *University College London*
INV76: mRNA therapy for liver inherited metabolic diseases

Valentina Poletti, *University of Padova*
INV77: Hematopoietic Stem Cell Gene Therapy for GM1 Gangliosidosis

Valeria Calbi, *SR-Tiget, Milan*
OR099: Atidarsagene autotemcel (lentiviral hematopoietic stem cell gene therapy) for late juvenile metachromatic leukodystrophy: preliminary results from a phase III clinical trial

Sarah Neuhaus, *Prevail Therapeutics, a wholly-owned subsidiary of Eli Lilly and Company*
OR100: Phase 1/2 dose-finding study to evaluate systemic administration of an AAV9-based gene therapy for peripheral manifestations of Gaucher disease: The Proceed Trial

Emily de los Reyes, *Nationwide Children's Hospital*
OR101: Gene therapy for CLN3, Juvenile neuronal ceroid lipofuscinosis, a promising therapy

Maria Escolar, *Forge Therapeutics*
OR102: REKLAIM, a Phase I/II clinical trial using a novel immune modulation strategy for systemic administration of FBX-101 (AAVrh10.GALC) after Umbilical Cord Blood Transplantation for the treatment of Infantile Krabbe Disease

11:00-13:00 · Meeting Room 2 · PARALLEL
SESSION 12c: Non-Viral Vectors / Nanotechnology II
Chairs: ***Marc Güell,*** *Pompeu Fabra University, Barcelona;* ***Gabor Tamas Szabo***, *BioNTech SE, Mainz*

Raymond Schiffelers, *Utrecht University*
INV78: Towards production of nucleic acid nanomedicines at the bedside

Hadi Valadi, *University of Gothenburg*
INV79: Utilizing extracellular vesicles for RNA transport and therapeutic applications

Lucia De Stefano, *Tigem, Naples*
OR103: mRNA-replacement therapy for Glycogen Storage Disease type 1b

Gilles Divita, DivinCell
OR104: A safe Peptide-based, lipid-free, nanoparticle platform for mRNA delivery and gene editing in the liver: application to Factor VIII rescue in hemophilia A and selective editing of PCSK9 gene to durably lowers cholesterol in mice.

Vanessa Hamann, *Hannover Medical School*
OR105: Alpharetrovirus-like particles for in vitro and vivo delivery of diagnostic and therapeutic RNAs

Peter Cabeceiras, *Nvelop Therapeutics, Cambridge, MA*
OR106: DLVR-M: a novel fully humanized particle for the efficient in vivo delivery of large gene-editing cargos to human cells

FRIDAY 25 OCTOBER

11:00-13:00 · Auditorium · PARALLEL

SESSION 12d: Regulatory

Chairs: ***Ilona Reischl,*** *EMA*

Patrick Celis, *EMA*
INV80: 15-years of CAT : what have we achieved, how have ATMP developers benefitted and what is CAT doing now to support ATMP development

Ilona Reischl, Kieran Breen, Emmely de Vries, *EMA*
INV81: CAT Guideline for investigational ATMPs in clinical trials: insights from the Regulator on quality, non-clinical and clinical requirements

Patrick Celis, *EMA*
INV82: EMA/CAT reflection on decentralised manufacturing for ATMPs

Panel discussion

13:00-13:30 · Meeting Room 1

SITGEC AGM

13:00-14:30 · Forum and Registration Foyer

LUNCH

13:30-14:15 · Satellite Room - M1

LUNCHTIME WORKSHOP: Am I an effective scientific communicator?

EuroGCT and the ECR Board Members will facilitate this workshop on effective communication, highlighting the strongest elements and offering tips for delivering impactful speeches.

Attendees must sign up in advance. Register at https://forms.office.com/e/YnrRv0EM3Y

14:30-16:45 · Plenary Hall · PLENARY

SESSION 13: Modeling Human Diseases to Advance Gene and Cell Therapies

Chairs: **Juan Bueren,** *CIEMAT/CIBERER/IIS. F. Jiménez Díaz, Madrid;* **Luigi Naldini,** *SR-Tiget, Milan;* **Alberto Auricchio,** *Tigem, Naples*

Manju Kurian, *University College London*
INV83: Modelling neurological disorders to advance gene therapies

Peter Campbell, *Wellcome Sanger Institute, Hinxton*
INV84: Somatic mutations in stem cell transplantation

Claire Henchcliffe, *University of California, Irvine*
INV85: Early clinical trials in developing a stem cell-based therapy in Parkinson's disease

Markus Grompe, *Oregon Health & Science University, Portland*
INV86: In vivo selection to enhance cell and gene therapy

Concluding Remarks

16:45-17:30 ·

Farewell drinks - See you in Sevilla