Congress Programme

ESGCT 29th Congress in
collaboration with BSGCT
11-14 October 2022 | EICC

Tuesday 11 October

Registration
08:30-09:00 Strathblane Hall

Education Session 1
09:00-10:45 Pentland/Sidlaw

Hildegard Büning, Hannover Medical School
Welcome

Anja Ehrhardt, *Private Universität Witten/Herdecke GmbH*
EDU01: The adenovirus vector platform: vector design and applications

Jude Samulski, *AskBio*
EDU02: AAV Gene Therapy: From Virus to Vector

Bernhard Gentner, *SR-Tiget, Milan*
EDU03: Retro- and lentiviral vectors for ex vivo gene therapy: an ongoing success story

Clinical Trials and Commercialisation Session 1: Clinical trials
09:00-10:50 Fintry

Claire Booth, *University College London* & **Juan Bueren,** *Ciemat, Madrid*
Welcome

Paula Walker, *Roche/Genentech*
CTC01: Good clinical practice: Clinical trials with gene therapies

Fiona Thistlethwaite, *The Christies NHS Foundation Trust*
CTC02: Clinical trial acceleration, the ATTC Network, a UK model with international applicability

Declan Noone, *European Haemophilia consortium, EMA PRAC*
CTC03: Putting the patient at the centre of clinical trial design

Maria Ester Bernardo, *SR-Tiget, Milan*
CTC05: Development of an ex-vivo GT platform for Lysosomal Storage Disorders with skeletal involvement

Coffee Break 10:45-11:15	**Coffee Break** 10:50-11:10
Education Session 2 11:15-12:45 Pentland/Sidlaw **Vincenzo Cerullo,** *University of Helsinki* **EDU04:** Oncolytic viruses and oncolytic vaccines **Paolo Martini,** *Moderna Therapeutics* **EDU05:** Messenger RNA encapsulated in Lipid Nonoparticles for tissue delivery **Julian Grunewald,** *TUM, Munchen* **EDU06:** New CRISPR technologies & clinical translation	**Clinical Trials and Commercialisation session 2: Regulatory & reimbursement challenges** 11:10-12:45 Fintry **Laura Beswick,** *Cell and Gene Therapy Catapult* **CTC06:** Planning for product development to generate necessary evidence for Health Technology Assessment **Jan Ohotski,** *Medspace* **CTC08:**Introduction to, and practical aspects of, the Clinical Trials Regulation **Claire Booth,** *UCL London* **CTC09:** The 'Agora' Initiative **Panel discussion: Overcoming barriers to commercialisation of ATMP's** **Anji Miller,** *LifeArc* **Marc Turner,** *SNBTS* **Maria Ester Bernardo,** *SR Tiget, Milan* **Owen Marks,** *Pfizer*

Main Congress Registration

12:00-14:00 Strathblane Hall

Plenary 1: ESGCT 2022 Opening Keynotes

14:00-16:30
Pentland Suite

Chairs: Andrew Baker, University of Edinburgh; Hildegard Büning, Hannover Medical School; Rafael Yáñez-Muñoz, Royal Holloway University of London

Andrew Baker, *University of Edinburgh*; **Hildegard Büning,** *Hannover Medical School*; **Rafael Yáñez-Muñoz,** *Royal Holloway University of London*
Welcome

Odile Cohen-Haguenauer, *Paris Diderot University*
INV01: 30 years of the ESGCT

Viviana Gradinaru, *CalTech*
INV02: Getting across barriers: Gene delivery across the blood-brain barrier for precise and minimally-invasive study and repair of nervous systems

Stuart Forbes, *University of Edinburgh*
INV03: Developing cell therapies for liver disease

Coffee Break

16:30 - 17:00

Parallel 1a: Gene & epigenetic editing

17:00-19:15
Pentland and Sidlaw

Chairs: Julian Grünewald, TUM, Munich; Alessia Cavazza, University College London

Miguel Esteban, *Guangzhou Institutes of Biomedicine and Health, Chinese Academy of Science*
INV08: Identification of mammalian progenitor cells at tissue and body scale

Parallel 1b: Haematopoietic stem cell therapies in immunodeficiencies and metabolic disorders

17:00-19:15
Fintry

Chairs: Claire Booth, University College London; Anne Galy, Genethon, Evry

Frank Staal, *Leiden University Medical Center*
INV04: Developing stem cell based gene therapy for RAG1 and RAG2 deficient SCID.

Parallel 1c: In vivo gene therapy & immune responses

17:00-19:15
Lomond Suite

Chairs: Gloria Gonzalez-Aseguinolaza, CIMA, Pamplona; Ivana Barbaric, University of Sheffield

Eric Kremer, *University of Montpellier*
INV06: Why are some adenovirus vectors poorly immunogenic?

Angelo Lombardo, *SR Tiget, Milan*
INV09: Exploiting targeted epigenome editing for therapeutic applications

Rajeev Rai, *UCL London*
OR10: CRISPR/Cas9 based disease modelling and functional correction of Interleukin 7 Receptor Severe Combined Immunodeficiency

Eleonora Pedrazzoli, *University of Trento*
OR11: CoCas9: a compact nuclease from the human microbiome for efficient and precise editing

Lucrezia della Volpe, *SR Tiget, Milan*
OR12: Inhibition of p38-MAPK counteracts DNA damage induced by ex vivo expansion of hematopoietic stem and progenitor cells for efficient genetic engineering

Julia Klermund, *University of Freiburg*
OR13: An in vivo CAST-Seq workflow identifies and quantifies off-target activity as well as chromosomal translocations in organs edited in vivo with CRISPR-Cas nucleases or nickases

Maria Silvia Roman Azcona, *University of Freiburg*
P425: Contemporary modulation of two major immune checkpoints expression in CAR T cells via hit-and-run epigenome editing

Sébastien Levesque, *Université Laval Quebec*
P371: MTOR as a selectable genomic harbor for CRISPR-engineered CAR-T cell therapy

Rosa Bacchetta, *Stanford School of Medicine*
INV05: Current Advances in the Gene therapy for IPEX syndrome

Francesca Ferrua, *SR Tiget, Milan*
OR01: Safety and clinical benefit of lentiviral haematopoietic stem and progenitor cell gene therapy in 23 patients with Wiskott-Aldrich Syndrome with up to 10.5 years of follow-up

Sameer Baha, *UCL, London*
OR02: Experience of genome editing patient haematopoietic stem cells to treat X-linked Agammaglobulinemia

Stuart Ellison, *University of Manchester*
OR03: Enhanced transduction and immunophenotyping demonstrates preclinical safety and efficacy of haematopoietic stem cell gene therapy for Mucopolysaccharidosis II (MPSII) using an IDS.ApoEII brain targeted therapy

Valentina Poletti, *University of Padova*
OR04: Preclinical development of an ex vivo gene therapy for Mucopolysaccharidosis type II

Ludovica Santi, *SR Tiget, Milan*
OR05: Skeletal damage and cross-correction in MPSIH HSPC-gene therapy

Hildegund Ertl, *The Wistar Institute, Philadelphia*
INV07: Immunotoxicity of high dose AAV vector gene transfer: can we develop protocols that allow for repeated dosing of lower and less toxic doses?

Greg Gojanovich, *AskBio*
OR06: Using a systems biology approach to unravel the Immunogenicity of AAV8 empty capsids in healthy volunteers

Kei Kishimoto, *Selecta BioSciences*
OR07: ImmTOR tolerogenic nanoparticles combined with Treg-selective IL-2 mutein induces massive expansion of antigen-specific regulatory T cells and synergistically inhibit formation of anti-AAV antibodies to high vector doses

Wolfgang Miesbach, *University Hospital Frankfurt*
OR08: Sustained factor IX activity levels and bleeding protection following etranacogene dezaparvovec administration in people with haemophilia B without and with adeno-associated virus serotype 5 neutralizing antibodies

Helena Costa Verdera, *SR Tiget, Milan*
OR09: Investigating the role of innate immune signaling in cell toxicity upon AAV-mediated gene transfer in hiPSC-derived CNS models

Ana Cavallo, *AstraZeneca*
P423: FaDe-Cas9: a fast degrading SpCas9 with reduced cellular immunity

Welcome Reception

19:00-20:00 Lennox Suite

Wednesday 12 October

Registration

07:30-08:30 Strathblane Hall

Parallel 2a: Vaccine technologies & infectious diseases

08:30-10:40
Pentland and Sidlaw

Chairs: Evangelia Yannaki, G.Papanikolaou Hospital, Thessaloniki; James Miskin, Oxford BioMedica

Jerome Custers, *Janssen, Johnson & Johnson*
INV10:

Darin Edwards, *Moderna Therapeutics*
INV11: Advancements in mRNA Science and Technology enabled a platform for rapid vaccine development against pandemic SARS-CoV-2

Ami Patel, *The Wistar Institute, Philadelphia*
INV12: From Pathogen to Protection: Engineering synthetic DNA-encoded vaccines and immunotherapies against emerging and pandemic viral pathogens

Derek Jantz, *Precision Biosciences*
OR14: Targeting the hepatitis B cccDNA with a sequence-specific ARCUS nuclease to eliminate Hepatitis B virus in vivo

Parallel 2b: Disease modelling in advanced therapies

08:30-10:40
Fintry

Chairs: Gerry McLachlan, University of Edinburgh; Giorgia Santilli, UCL, London

Patapia Zafeiriou, *University of Goettingen*
INV13: Human bioengineered neuronal organoids as preclinical models for gene therapy

Meritxell Huch, *MPI-CBG Dresden*
INV14: Exploiting organoids as disease models

Ranjita Devi Moirangthem, *Institut Imagine, Paris*
OR16: Ex Vivo Immunotherapeutic NK Cell Production From Cord Blood Or Mobilized Peripheral Blood CD34+ Cells Using Notch Ligand Delta-Like 4 Culture System

Els Verhoeyen, *University of Nice*
OR17: Nanoblades allow high-level genome editing in organoids

Parallel 2c: Metabolic diseases

08:30-10:40
Lomond Suite

Chairs: Fulvio Mavilio, Orchard Therapeutics; Fatima Bosch, UAB Barcelona

Andrea Annoni, *SR-Tiget, Milan*
INV15: Inverse vaccination strategies for the induction of Ag-specific immune tolerance in autoimmune diseases

Paolo Martini, *Moderna Therapeutics*
INV16: Messenger RNA therapy is a platform therapeutic for the treatment of Rare Genetic Disorders

Tarekegn Geberhiwot, *University of Birmingham*
OR20: Final safety and efficacy of a phase 1/2 trial of DTX301 in adults with late-onset ornithine transcarbamylase deficiency (OTCD)

P. Deegan, *Addenbrooke's Hospital, Cambridge*
OR21: Preliminary results of STAAR, a Phase I/II study of isaralgagene civaparvovec (ST-920) gene therapy in adults with Fabry disease and long-term follow-up

Allan Scarpitta, *University of Rouen*
OR15: AAV-mediated induction of immunogenic cell death to promote anti-tumor immune responses

Frederick Rothemejer, *Aarhus University*
P500: HIV-resistant anti-HIV CAR T cells as a functional cure

Jacqueline Doms, *ART-TG, Inserm France*
P504: A non-viral CRISPR/Cas9 gene-editing approach to express anti-HIV broadly-neutralizing antibodies in human B cells for HIV immunotherapy

Myriam Lemmens, *Novartis*
OR18: Identification of marker genes to monitor residual iPSCs in iPSC-derived products

Liam Kempthorne, *UCL London*
OR19: Utilising CRISPR-Cas13 systems to target frontotemporal dementia and amyotrophic lateral sclerosis-causing C9orf72 repeat expansion-containing RNA

Caterina Gomes, *Ibet Lisboa*
P350: Human stem-cell based models to study innate immunity and neuroinflammation in the central nervous system

Myriam Lemmens, *Novartis*
P352: Identification of marker genes to monitor residual iPSCs in iPSC-derived products

Loukia Touramanidou, *UCL London*
OR22: In vivo lentiviral gene therapy restores ureagenesis in the urea cycle defect argininosuccinic aciduria

Randy Chandler, *National Human Genome Research Institute*
OR23: Gene Therapy to Treat Methylmalonic and Propionic Acidemia Using the Novel Adeno-associated Viral Capsid 44.9

Estera Rintz, *University of Gdansk*
P674: Adeno-associated virus vector combination gene therapy with C-type natriuretic peptide and GALNS enzyme in Mucopolysaccharidosis IVA mouse model

Bethan Critchley, *UCL London*
P537: Targeting the blood brain barrier for haematopoietic stem cell gene therapy in neurological lysosomal storage disorders

Break

10:40-11:10

Plenary 2: Clinical Trials

Orchard therapeutics

11:10-13:15 Pentland Suite

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

Peter Marinkovich, *Stanford University School of Medicine*
INV17: Topical gene therapy for dystrophic epidermolysis bullosa, results of a phase 3 trial

Bernhard Gentner, *SR-Tiget, Milan*
INV18: Genetically-modified hematopoietic stem cells as a one-time, systemic treatment for non-hematologic disorders.

Nicola Brunetti-Pierri, *Tigem, Naples*
INV19: Liver-Directed Gene Therapy for Mucopolysaccharidosis Type VI

Don Kohn, *University of California, Los Angeles*
INV20: Interim Results from an ongoing Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)

Pratima Chowdary, *UCL, London*
INV21: A novel adeno-associated virus (AAV) gene therapy (FLT180a) achieves normal FIX activity levels in severe Hemophilia B (HB) patients (B-AMAZE study)

Lunch

13.15-15:15

Lunchtime sponsor symposium: Viralgen

13:45-14:45
Pentland

VIRALGEN

Cesar Trigueros, *Viralgen, San Sebastian*
A Platform Approach For Adeno-Associated Virus: Manufacturing To Support Gene Therapy Product.

Lunchtime sponsor symposium: Optimizing AAV manufacturing for commercial gene therapy production

ThermoFisher SCIENTIFIC

13:45-14:45
Fintry

Chairs: Roland Leathers, Strategic Alliances Manager, Thermo Fisher Scientific

Helena Meyer-Berg, *AAV Platform Manager Project Developer Cell & Gene Therapy Sirion Biotech*

Toke Jost Isaken, *Senior Scientist, RNA & Gene Therapies, Global Research Technologies, Novo Nordisk A/S*

Lunchtime sponsor symposium: PTC Therapeutics

PTC THERAPEUTICS

14:00-15:00
Sidlaw

Philippe Moyen, *PTC Therapeutics*
PTC Therapeutics: partners for pDNA and clinical AAV Vector manufacturing

Dr Vincent D'Hardemare, Service de neurochirurgie adultes – Hospital Fondation Rothschild
Intraputaminal gene therapy for AADC deficiency: Surgery and outcomes

Philippe Moyen and Dr Vincent D'Hardemare
Panel discussion

Parallel 3a: CNS and sensory diseases I

15:15-17:10
Pentland / Sidlaw

Chairs: Alberto Auricchio, Tigem, Naples; Rajvinder Karda, UCL London

Krystof Bankiewicz, *University College London*
INV26: MR-guided midbrain administration of AAV2AADC in patients with AADC deficiency results in clinical improvement

Nicole Déglon, *Centre Hospitalier Universitaire Vaudois (CHUV), Lausanne*
INV27: Genetic engineering to the rescue of Huntington's disease

Amy Geard, *UCL London*
OR30: AAV9-mediated gene therapy in a knock-in mouse model of infantile neuroaxonal dystrophy

M Kaplitt, *Weill Cornell Medical College, NYC*
OR31: Gene therapy in APOE4 homozygote Alzheimer's disease – interim data

Kevin Nash, *University of South Florida*
OR32: Reelin Gene Therapy for Fragile X Syndrome

Sergi Verdes, *UAB Barcelona*
P184: Gene therapy for ALS by specifically overexpressing a pleiotropic chronokine, secreted α-Klotho, in skeletal muscles

Kathrin Meyer, *Nationwide Children's Hospital*
P185: A Novel AAV Gene Therapy for Rett Syndrome through Reactivation of the Silent X Chromosome

Parallel 3b: Advances in Haemophilia A

15:15-17:10
Fintry

Chairs: Christos Georgiadis, UCL London; Hildegund Ertl, The Wistar Institute, Philadelphia

Kevin Eggan, *BioMarin*
INV22: Molecular Characterization of Recombinant AAV5 Encoding FVIII After Human Administration

Antonia Follenzi, *Univesita del Piemonte Orientale, Novara*
INV23: Targeting FVIII expression to liver endothelium

Felix Lansing, *Dresden University*
OR24: Fusion of Designer recombinases for efficient and specific correction of a Factor VIII genomic inversion

Federica Esposito, *Tigem, Naples*
OR25: AAV-mediated homology-independent targeted integration leads to sustained secretion of therapeutic proteins from new-born liver.

Sara Deola, *Sidra Medicine*
OR26: Assessment of FVIII in the hematopoietic system: UM171-expanded CD31+ monocytes show the highest GT potential to correct HA

Parallel 3c: Advanced therapies for solid tumors

15:15-17:10
Lomond Suite

labcorp Drug Development

Chairs: Cristina Fillat, IDIBAPS, Barcelona; Alan Parker, Cardiff University

Marta Alonso, *University of Navarra*
INV24: Virotherapy for pediatric brain tumors: more than a hope

Guy Ungerechts, *NCT/DKFZ, Heidelberg*
INV25: Immunovirotherapy – Clinical Translation

Thomas Kerzel, *SR Tiget, Milan*
OR27: IFNalpha by in vivo-engineered macrophages abates liver metastases and triggers counter regulatory responses limiting treatment efficacy

Alicia Teijeira Crespo, *University of Cardiff*
OR28: Development of a tumour selective precision immunovirotherapy expressing immune checkpoint inhibitors targeting LAG3

Alessia Potenza, *SR Tiget, Milan*
OR29: Harnessing CD39 for the treatment of colorectal cancer and liver metastases by engineered T cells

Ryan Murray, *Northeastern University*
P376: Multiplex base editing protects allogeneic solid tumor targeting CAR-T cells from inhibition by extracellular adenosine in the tumor microenvironment

		Owen Moon, *InstilBio* **P382:** Antitumor activity of T cells expressing a novel anti-folate receptor alpha (FRα) costimulatory antigen receptor (CoStAR) in a human xenograft murine solid tumor model and implications for in-human studies

Break

17:10-17:40

Plenary 3: AAV safety

17:40-19:30 Pentland Suite

Chairs: Hildegard Büning, Hannover Medical School; Christopher Mann, Asphalion Therapeutics

Terry Flotte, *University of Massachusetts*
INV28: Overview of Clinical Gene Therapy with AAV from the Safety Perspective

Denise Sabatino, *Children's Hospital of Philadelphia*
INV29: Current understanding of AAV integration and the potential risk of AAV-associated insertional mutagenesis

Juliette Hordeaux, *University of Pennsylvania*
INV30: AAV-related toxicities in nonhuman primates

Round Table
Carla Herberts, Hildegard Büning, Terry Flotte, Denise Sabatino, Juliette Hordeaux, Declan Noone

Poster session 1

19:30-21:00
Cromdale Hall

Posters with an ODD number

Thursday 13 October

Registration

07:30-08:30 Strathblane Hall

Parallel 4a: Gene and epigenetic editing II

08:30-10:45
Pentland / Sidlaw

Chairs: Francisco Martin, Genyo, Granada; Paula Rio, Ciemat, Madrid

Claudio Mussolino, *University of Freiburg*
INV35: Strategies to improve precision and safety of human cells manipulation.

Raffaella Di Micco, *SR-Tiget, Milan*
INV36: Mechanistic insights to advance hematopoietic stem cell based gene therapies

Mariacarmela Alloca, *Editas Medicine*
OR43: A Mutation-Independent CRISPR/Cas9-based 'Knockout and Replace' Strategy to Treat Rhodopsin-Associated Autosomal Dominant Retinitis Pigmentosa

Alice Rovai, *Hannover Medical School*
OR44: In vivo adenine base editing corrects the HFE C282Y mutation and improves iron metabolism in hemochromatosis mice

Martino Cappellutti, *SR Tiget, Milan*
OR45: Durable silencing of Pcsk9 by in vivo hit-and-run epigenome editing.

Parallel 4b: CNS & sensory II

08:30-10:45
Fintry

Chairs: Simon Waddington, UCL London; Robin Ali, Kings College London

Nathalie Cartier, *AskBio, Paris*
INV33: Pathway Gene therapy for Huntington disease : A Phase I/II Dose-Finding study to Evaluate BV-101 Striatal Administration in Adults with Early Manifest Hutington's disease

Hinrich Staecker, *University of Kansas Medical Center*
INV34: Leveraging cochlear implantation to accelerate the translation of inner ear gene therapy

Larissa Nassauer, *Hannover Medical School*
OR38: Development of novel gene therapeutic approaches for protection from drug-induced ototoxicity

Rita Milazzo, *Altheia Science s.r.l*
OR39: Clinical benefit of PD-L1 hematopoietic stem and progenitor cell gene therapy in an animal model of multiple sclerosis

Sophia Millington Ward, *Trinity College Dublin*
OR40: AAV-delivered NDI1 improves mitochondrial function and provides benefit in AMD models

Parallel 4c: Advanced therapies with CAR-T cells

08:30-10:45
Lomond Suite

Chairs: Ulrike Koehl, Fraunhofer IZI, Leipzig; Alessia Potenza, SR Tiget, Milan

Monica Casucci, *SR-Tiget, Milan*
INV31: Exploiting N-glycosylation blockade to boost CAR-T cell efficacy in solid tumours

Maria Themeli, *Amsterdam University Medical Center*
INV32: Multi-targeting and combinatorial costimulation to improve CAR T cell therapy

Isabel Lane, *Harvard Medical School*
OR33: Genetically retargeting E3 ligases to enhance CAR T cell therapy

Celine Rocca, *Genethon, France*
OR34: Immunotherapy treatment with FAP-specific CAR-T cells can reduce skeletal muscle fibrosis in a murine model of Duchenne muscular dystrophy

Pedro Luis Justicia Lirio, *GENYO, Granada*
OR35: Generation of first-in-class doxycycline-inducible IL-18 releasing CAR-T cells targeting solid tumors

Arianna Moiani, *Cellectis Therapeutics*
OR46: Non-viral DNA delivery associated to TALEN® gene editing leads to highly efficient correction of sickle cell mutation in long-term repopulating hematopoietic stem cells

Paula Rio, *Ciemat Madrid*
P144: Therapeutic Base and Prime Editing in Fanconi Anemia Hematopoietic Stem and Progenitor Cells

Marco Luciani, *SR Tiget, Milan*
P427: Epigenome-editing strategies to enhance oligodendroglial differentiation from human induced pluripotent stem cells

Younbok Lee, *Kings College London*
OR41: Intra-thalamic delivery of AVB.PGRN rescues pathology in Grn null mice and achieves widespread cortical expression in a large animal model

Barbara Bettegazzi, *University Vita Salute San Raffaele Milan*
OR42: A combinatorial gene therapy for temporal lobe epilepsy based on NPY and one of its receptors

Christos Georgiadis, *UCL, London*
OR36: CRISPR-coupled CAR engineering of universal donor T cells for paediatric B-ALL

Mateo Doglio, *SR Tiget, Milan*
OR37: CAR-Tregs for Systemic Lupus Erythematosus

Break

10:45-11:15

Plenary 4: Presidential symposium

In memoriam: Dr Manfred Schmidt

11:15-13:25 Pentland Suite

Chairs: Hildegard Büning, Hannover Medical School; Juan Bueren, CIEMAT, Madrid

Hildegard Büning, *Hannover Medical School*
Presidential address

Eugenio Montini, *SR-Tiget, Milan*
INV37: New frontiers in genotoxicity testing and clonal tracking methodologies

Presentation of the ESGCT Outstanding Achievement Award

Human Gene Therapy

Christof von Kalle, *Charité Berlin*
Manfred Schmidt, In Memoriam

Presentation of the Special Anniversary Award

Presentation of the Founders Award

Jude Samulski, *AskBio*

Award ceremony for 2021 awards and announcement of Travel Grant winners

Eliana Ruggiero, *SR-Tiget, Milan*
INV39: The TCR Therapy Revival: Gene-Edited WT1-Specific T Cells Treat Leukemia and Solid Tumours

Raul Torres, *CNIO Madrid*
INV40: Genome engineering for cancer applications

Lunch

13.25-15:30

Enhanced support to academic developers of ATMPs

13:45-14:45
Pentland

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patrick Celis, *EMA*
Academic ATMP development support pilot at the European Medicines Agency

Alessandro Aiuti, *SR Tiget, Milan*
The view of an academic developer

Lunchtime sponsor symposium: A lightbulb moment for gene editing

13:45-14:45
Fintry

MaxCyte®

Chairs: Lesley Eschinger, MaxCyte

Elena Stoyanova, *Touchlight Genetics*

Lunchtime sponsor symposium: Unleashing the Allogeneic Potential: Applying Separation Technologies in Large Scale Manufacture to Achieve Off-the-Shelf Products in Advanced Therapies

13:45-14:45
Sidlaw

SARTORIUS

Chair: Noushin Dianat, External Collaborations Manager, Sartorius

Guest Speaker
Lior Raviv, *Vice-President & CTO, Pluri*

Speaker
Michal Szelwicki, *Product Specialist, Sartorius*

Parallel 5a: AAV next generation vectors

15:30-17:30
Pentland /Sidlaw

Chairs: Jude Samulski, AskBio; Terry Flotte, University of Massachusetts Medical School

Leszek Lisowski, *CMRI, University of Sydney*
INV47: Designer therapeutics: pushing the limits of AAV capsid engineering.

Ken Macnamara, *AskBio*
INV48: PromPT: data-driven vector design for precise control of gene expression

Waldemar Schäfer, *University Medical Center Hamburg*
OR55: Nanobodies as versatile ligands to retarget AAV

Jennifer Marx, *Hannover Medical School*
OR56: Capsid-engineered adeno-associated virus (AAV) vector for neurotrophin gene therapy in inner ear disease

Guangping Gao, *UMass Chan Medical School*
OR57: Endogenous human SMN1 promoter-driven gene replacement improves the efficacy and safety of AAV9-mediated gene therapy for spinal muscular atrophy (SMA) in mice

Jacqueline Bogedein, *LMU Munich*
OR58: Mutagenesis of capsid surface residues for improvement of AAV vectors with retinal tropism

Parallel 5b: Developments in manufacturing and scale up

15:30-17:30
Fintry

Chairs: Mustafa Munye, Complement Therapeutics; Beata Surmacz-Cordle, GSK

Brian Mullan, *Yposkesi, Paris*
INV43: Cell & Gene therapy manufacturing – current state and future developments

Ulriche Köhl, *Fraunhofer IZI, Leipzig*
INV44: Manufacturing and quality control of CAR effector cells.

Eduard Ayuso, *Dinamiqs*
INV45: Seamless AAV manufacturing from proof-of-concept to clinical applications

Sophie Mountcastle, *MRC* & **Anji Miller,** *LifeArc*
INV46: Supporting translation of gene therapies through manufacturing infrastructure and a new research fund for early-phase clinical trials

Panagiota Cristofi, *University of Patras*
OR51: Characterization and GMP manufacturing of epigenetically reprogrammed HLA-G expressing, T regulatory cells (iG-Tregs)

Fabrizio Benedicenti, *SR Tiget, Milan*
OR52: Sonication Linker Mediated-PCR (SLiM-PCR), an efficient method for quantitative retrieval of vector integration sites

Yuriko Makino, *Tosoh Corp*
OR53: Application of novel AAV purification and analysis column with Adeno-Associated Virus Receptor (AAVR)

Parallel 5c: Blood diseases: Haematopoietic cell disorders

15:30-17:30
Lomond Suite

Chairs: Maria Esther Bernardo, SR Tiget, Milan; Jose Carlos Segovia-Sanz, Ciemat, Madrid

Juan Bueren, *CIEMAT, Madrid*
INV41: Preliminary Conclusions of the Phase I/II Gene therapy Trial in Patients with Fanconi Anemia-A

Punam Malik, *Cincinnati Children's Hospital*
INV42: Gene therapy for hematopoietic disorders: Improving Engraftment of Genetically Modified Cells

Rafi Emmanuel, *EmendoBio, Israel*
OR47: EMD-101, an autologous, allele-specific gene-edited hematopoietic stem cell product, for treating ELANE-mediated severe congenital neutropenia

Panagiotis Antoniou, *Institut Imagine, Paris*
OR48: Sharpening the adenine and cytosine base editing outcome in hematopoietic stem cells

Oscar Quintana Bustamante, *Ciemat, Madrid*
OR49: Correction of Congenital Dyserythropoietic Anemia Type II using Lentiviral Gene Therapy

Samantha Scaramuzza, *SR Tiget, Milan*
OR50: Reconstitution of Humoral and Cellular Immunity in Thalassemic Patients Treated with HSC LV-mediated Gene Therapy Following Myeloablation

	Mohamed Ashrafali, *Biomarin, Pharmaceutical* **OR54:** Vector genome loss and epigenetic modifications impact long-term transgene expression of AAV5 vectors produced in mammalian HEK293 and insect Sf cells	

Break and Poster session II

17:30-19:15 Cromdale Hall

Posters with an EVEN number

The Molecular Mingle

19:30 National Museum of Scotland.

Friday 14 October

Registration

08:30-09:00 Strathblane Hall

Parallel 6a: Gene & epigenetic editing III

09:00-10:30
Pentland / Sidlaw

Chairs: Toni Cathomen, University of Freiburg; Axel Schambach, Hannover Medical School

Luigi Naldini, *SR-Tiget, Milan*
INV54: Transformative Approaches to Genetic Engineering of Hematopoiesis based on Gene Editing and RNA Transfection

Ana Hinckley Boned, *Genyo, Granada*
OR61: A negative regulatory region in WAS intron 1 controls megakaryocytic differentiation

Enrico Surace, *University of Naples*
OR62: Therapeutic changes of cis and trans regulatory elements

Lei Lei, *University of Freiburg*
OR63: Base editing in hematopoietic cells corrects the disease underlying intronic mutation in a mouse model of familial hemophagocytic lymphohistiocytosis

Samuele Ferrari, *SR-Tiget*
OR64: Assessing Stealth and Sensed Base and Prime Editing in Human Hematopoietic Stem/Progenitor Cells

Parallel 6b: Novel advances in gene and cell therapy in the US

ASGCT American Society of Gene + Cell Therapy

09:00-10:30
Fintry

Chairs: Rayne Rouce, Texas Children's Hospital

Stephen Russel, *Mayo Clinic, Rochester*
INV49: Clinical experience with VSV-IFNb-NIS, an oncolytic vesicular stomatitis virus

Rayne Rouce, *Texas Children's Hospital*
INV50: Next Level Cell Therapy": New Targets, New Sources, New Possibilities...New Problems

Rebecca Larson, *Massachusetts General Hospital and Harvard Medical School*
INV51:

Parallel 6c: CNS and sensory III

09:00-10:30
Lomond Suite

Chairs: Giulia Massaro, UCL London; Nicole Déglon, University of Lausanne

Stephan Pollard, *University of Edinburgh*
INV52: Synthetic super-enhancers enable cell type-selective expression of anticancer payloads for viral gene therapy

Stefanie Schorge, *University College London*
INV53:

Pasqualina Colella, *Stanford University*
OR59: Novel conditioning enables high and stable repopulation of macrophage/microglia niches by wild type and genome-edited hematopoietic cells.

Annita Montepeloso, *Harvard Medical School*
OR60: Targeting CX3CR1 Gene To Improve Microglia Reconstitution And Transgene Delivery Into The CNS Upon Hematopoietic Stem And Progenitor Cell Transplant

Break

10:30-11:00

Parallel 7a: Cardiovascular diseases

11:00-13:00
Pentand / Sidlaw

Chairs: Stuart Nicklin, University of Glasgow; Seppo Ylä-Herttuala, University of Eastern Finland, Kuopio

Wolfram Zimmermann, *University Medical Center Göttingen*
INV63: Tissue Engineered Heart Repair from Bench to Bedside

Andrew Baker, *University of Edinburgh*
INV64: Modification of vein graft failure by gene therapy and RNA therapeutics

Roger Hajjar, *Ring Therapeutics, Cambridge MA*
INV65: Clinical Cardiac Gene Therapy Comes of Age

Ignacio Perez de Castro, *Instituto de Salud Carlos III*
OR67: Study of the potential of gene therapy approaches for the treatment of LMNA-related congenital muscular dystrophy

Izabella Kraszewska, *Jagiellonian University*
OR68: Identification of barriers to AAV transduction in the heart: focus on receptor availability and intracellular vector processing

Parallel 7b: Nanoparticles and biomaterials

11:00-13:00
Fintry

Chairs: Alexander Baker, Accession Therapeutics, Oxford; Els Verhoeyen, Université de Nice, Université de Lyon

Zoltan Ivics, *Paul Ehrlich Institute*
INV60: Advances in Sleeping Beauty transposon-based therapeutic cell engineering

Ivana Trapani, *Tigem, Naples*
INV61: Tackling diseases due to mutations in large genes using AAVs

Helder Santos, *University of Helsinki*
INV62:

Keittisak Suwan, *Imperial College London*
OR65: Chimeric phagemid/AAV for targeted gene delivery and cancer immunotherapy

Michele Palamenghi, *University of Modena e Reggio Emilia*
OR66: Gamma-retroviral hotspots integration in human primary keratinocytes: potential implication for clinical application

Parallel 7c: EMA Committee for Advanced Therapies - Regulatory Aspects of ATMPs

11:00-13:00
Lomond Suite

Chairs: Martina Schuessler Lenz, PEI; Ilona Reischl, AGES - MEA

Martina Schuessler-Lenz, *PEI Langen*
INV57: Regulatory and scientific aspects of ATMPs-– an update from the Committee for Advanced Therapies

Barbara Bonamassa, *AIFA, Rome*
INV58: Regulatory aspects of gene-editing based ATMPs

Claire Beuneu, *AFMPS, Brussels*
INV55: Current status of the ICH S12 guideline on biodistribution studies for gene therapies

Carla Herberts, *CBG Meb, Amsterdam*
INV56: Regulatory considerations on integrational events of recombinant AAV-based gene therapy

Ilona Reischl, *EMA Amsterdam*
INV59: Hot topics for ATMP developers

Round table discussion

Break

13:00-14:30

The ERC's programme and widening European participation: What's on the horizon?

13:30-14:15
Fintry

Chairs: Janka Mátrai, ERC

Join our ERC session to get funding updates and advice from experts. Participate in the round table discussion and ask your questions on ERC's newest funding opportunities and actions towards widening European participation and closing the currently existing success gap. The session is organised and chaired by Janka Mátrai, and kindly supported by two distinguished guest scientists, Joanna Madzio and Zoltán Ivics

Plenary 5: Novel approaches on cell and gene therapy

14:30-17:00 Pentland Suite

Chairs: Hildegard Büning, Hannover Medical School; Juan Bueren, Ciemat Madrid; Rafael Yáñez-Muñoz, Royal Holloway University of London

Luca Biasco, *University College London/Sana Biotechnology*
INV66: Novel cellular targets and delivery strategies for hematopoietic stem/progenitor cell gene therapy

Mathias Vormehr, *BioNTech*
INV67: mRNA vaccines for the treatment of cancer

Bobby Gaspar, *Orchard Therapeutics*
INV68: Ending the devastation caused by severe genetic diseases through HSC gene therapy and newborn screening: a case study in metachromatic leukodystrophy

Claire Booth, *University College London*; **Alessandro Aiuti,** *SR-Tiget, Milan*
INV69: Access to gene therapies for rare diseases - it's time to act

Rafael Yáñez-Muñoz, *Royal Holloway University of London*
BSGCT Fairbairn Award

Hildegard Büning, *Hannover Medical School*
Closing words

Closing drinks

17:00 Strathblane Hall