**TUESDAY 24 OCTOBER**

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<thead>
<tr>
<th>Time</th>
<th>Location</th>
<th>Session/Event</th>
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<tr>
<td>12:00-14:00</td>
<td>Gare Maritime</td>
<td>Registration</td>
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<tr>
<td>14:00-16:30</td>
<td>Shed 2A</td>
<td>Plenary Session 1: ESGCT 2023 Opening</td>
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<td>Chairs: Juan Bueren, CIEMAT/CIBERER/IS, E. Jiménez Díaz, Madrid; Thierry VandenDriessche, Free University of Brussels; Victor Van Beusechem, Amsterdam University Medical Center; Els Verhoeyen, University of Nice</td>
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<td></td>
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<td>Welcome</td>
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<td>Inv01: Antigen sensitivity, logic gating and persistence of CAR T cells.</td>
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<td>Inv02: Hemophilia gene therapy: advances and challenges.</td>
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<td>Inv03: Successful clinical use of gene addition and transcript knockdown in SCID-X1 and Sickle Cell Disease</td>
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<td>Inv04: Exploration of Biological Diversity</td>
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<td>17:00-19:00</td>
<td>Shed 2A</td>
<td>Parallel Session 2a: Gene transfer and genome editing for cancer immunotherapy</td>
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<td>Chairs: Chiara Magnani, University of Zurich; Sonia Guedan, IDIBAPS, Barcelona</td>
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<td>Inv05: From TCRs to engineered T cell products</td>
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<td>Inv06: TCR and inhibitory receptor genome editing</td>
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<td>Inv07: Deliver to the liver: towards one-and-done gene therapies for defects of hepatic metabolism.</td>
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<td>17:00-19:00</td>
<td>Shed 2B</td>
<td>Parallel Session 2b: Lentiviral &amp; integrative vectors</td>
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<td>Chairs: Els Verhoeyen, University of Nice; Axel Schambach, Hannover Medical School</td>
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<td>Inv07: Understanding intracellular Innate immunity and how to apply it to infection, cancer and gene therapies</td>
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<td>Inv08: Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency</td>
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<td>Or05: Acquisition of somatic mutations after hematopoietic stem cell gene therapy varies among cell lineages and is modulated by vector genotoxicity and the activity of key cellular</td>
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<td>Or06: In vivo hematopoietic stem cell gene therapy using BaEVRLess-pseudotyped retroviral vectors</td>
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### TUESDAY 24 OCTOBER

#### 17:00-19:00 ∙ Maison de la Poste ∙ PARALLEL

**SESSION 2c: CNS & sensory diseases I**

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

**Stylianos Michalakis,** University Hospital LMU Munich  
**INV09:** Intravitreal gene therapy for inherited retinopathies

**Dimitri Kullmann,** University College London  
**INV10:** Gene therapy for focal pharmacoresistant epilepsy

**Jennifer Hodges,** Sangamo Therapeutics, Inc

**OR09:** Zinc Finger Activators restore normal gene and protein expression in a mouse model of SCN2A haploinsufficiency

**Federica Espsito,** Tigem, Naples  
**OR10:** Human RHO editing for treatment of autosomal dominant Retinitis Pigmentosa

**François Piguet,** TiDU GENOV, ICM, Paris  
**OR11:** Development and validation of a novel Adeno-Associated Viral gene therapy for Mucopolysaccharidosis IIIb (MPSIIIb)

**Vanessa Zancanella,** Research and Development, uniQure biopharma B.V., Amsterdam  
**OR12:** Gene therapy for C9orf72-ALS reduces RNA toxicity and ameliorates behavioral phenotype in ALS mouse model

#### 17:00-19:00 ∙ BEL ∙ PARALLEL

**SESSION 2d: From research to clinical trials: exploring opportunities for scientific-regulatory support**

**Chairs:** Marcos Timon, Agencia Española de Medicamentos y Productos Sanitarios; Martina Schuessler-Lenz, Paul Ehrlich Institute, Langen

**Martina Schuessler-Lenz,** Paul Ehrlich Institute, Langen  
**Introduction:** Scientific and regulatory support in Europe – an overview

**Wolfram Zimmermann,** University Medical Center Göttingen  
**INV11:** How to develop my research candidate towards a clinical trial. Experience of an academic developer.

**Christophe Lahorte,** Federal agency for medicines and health products (FAMHP); Andre Berger, Paul Ehrlich Institute, Langen  
**INV12:** How we support ATMP developers – the National Competent Authorities’ view (Belgium and Germany)

**Johanna Reul,** Paul Ehrlich Institute, Langen  
**INV13:** CMC challenges in the development of gene therapies and how to address them – regulatory aspects

**Marcos Timon,** Agencia Española de Medicamentos y Productos Sanitarios  
**INV14:** Innovative approaches in the manufacturing of ATMPs – regulatory aspects

**Claire Beuneu,** Federal agency for medicines and health products (FAMHP)  
**INV15:** How to get a clinical trial approved in the EU – pitfalls and opportunities

#### 19:00-20:30 Shed 1

**WELCOME RECEPTION**
### WEDNESDAY 25 OCTOBER

**REGISTRATION**

07:30-08:30 - Gare Maritime

**SESSION 3a: Gene editing: Technology development**

*Chairs: Adi Barzel, Tel Aviv University; Matt Porteus, Stanford University*

- **Toni Cathomen,** University of Freiburg
  - **INV16:** CRISPRing the genome: important lessons from evaluating on- and off-target effects

- **Anna Cereseto,** University of Trento
  - **INV17:** Maximizing the exploitation of a massive metagenomic data for the advancement genome editing tools

- **Denise Klatt,** Dana Farber/Boston Children’s Cancer and Blood Disorders Center, Harvard Medical School, Boston, MA
  - **OR13:** Alpha-retrovirus-based virus-like particles for efficient CRISPR-Cas9 delivery into hematopoietic stem cells

- **Hui Yang,** HuidaGene Therapeutics Co., Ltd., Shanghai
  - **OR14:** Programmable deaminase-free base editors for G and T base editing by engineered glycosylase

- **Massoud Nasri,** University Hospital Tübingen
  - **OR15:** MILESTONE, A universal CRISPR/Cas9-mediated genome editing strategy for ELANE-related severe congenital neutropenia

- **Francesco Puzzo,** Stanford University
  - **OR16:** DNA/RNA hybrids as double edge sword: r-loops can influence the efficiency of AAV-mediated genome editing and undesired vector integrations in vivo

08:30-10:30 - Shed 2A - PARALLEL

**SESSION 3b: AAV, non-integrative vectors I**

*Chairs: Leszek Lisowski, CMRI, University of Sydney; Hildegard Büning, Hannover Medical School*

- **Dirk Grimm,** University of Heidelberg
  - **INV18:** Small steps for viruses, giant leaps for mankind: Latest advances in AAV capsid engineering

- **Daniela Cesana,** San Raffaele Scientific Institute
  - **INV19:** AAV Integration in preclinical models of gene therapy

- **Hildegard Büning,** Hannover Medical School
  - **OR17:** In Vivo Targeting of HSC by Capsid-Engineered AAV Vectors

- **Ai Vu Hong,** Genethon, UMR-S951, Inserm, Univ Evry, Université Paris Saclay, EPHE
  - **OR18:** An Integrin-targeting AAV developed by a novel computational rational design methodology presents an improved targeting to the skeletal muscle and reduced toxicity.

- **Luca Zinser,** Paul-Ehrlich-Institut, Langen
  - **OR19:** DART-AAVs enable specific transduction of murine and human CD8 T cells for in vivo gene therapy

- **Florencia Haase,** Children’s Medical Research Institute, Sydney
  - **OR20:** Directed evolution of novel AAV-capsid variants for efficient and specific targeting of primary human Schwann cells

08:30-10:30 - Maison de la Poste - PARALLEL

**SESSION 3c: Gene therapy for metabolic diseases at preclinical stage**

*Chairs: Gloria Gonzalez-Aseguinolaza, CIMA, Pamplona, Giuseppe Ronzitti, Genethon and UMR_951, Evry*

- **Fatima Bosch,** Universitat Autonoma de Barcelona
  - **INV20:** Skeletal Muscle-Directed Gene Therapy for NASH, Insulin Resistance and Obesity

- **Nick Weber,** Vivel Therapeutics
  - **INV21:** Advances and pitfalls in AAV therapy targeting inherited cholestasis

- **Randy Chandler,** National Human Genome Research Institute
  - **OR21:** Rescue of a Lethal Murine Model of Methylmalonic Acidemia After AAV Delivery of Liver Specific and Global Piggybac Transposase Systems

- **Yvonne, Aratyn-Schaus,** Beam Therapeutics, Inc
  - **OR22:** A single, systemic administration of BEAM-301 mitigates fasting hypoglycemia one year after dosing in a transgenic mouse model of glycogen storage disease type Ia

- **Elena Barbon,** SR Tigel, Milan
  - **OR23:** Lentiviral-based liver gene therapy provides long-term efficacy and safety, global restoration of liver pathology and therapeutic benefit in kidney and brain, in a mouse model of methylmalonic acidemia.

- **Asma Naseem,** Department of Infection, Immunity & Inflammation, UCL Great Ormond Street Institute of Child Health, University College London
  - **OR24:** Prenatal in vivo base editing for the treatment of Krabbe disease
### Wed 25 October

#### 08:30-10:30 ∙ Bel ∙ Parallel

**SESSION 3d: Innate barriers to nucleic acid delivery**

*Chairs: Anna Kajaste, SR-Tiget, Milan; Rein Verbeke, Gent University*

- **INV22:** Systemic and local immune responses to ocular gene therapy
  - Deniz Dalkara, Institut de la Vision, Paris

- **INV23:** Lipid Nanoparticles for Overcoming Biological Barriers to mRNA Delivery
  - Michael Mitchell, University of Pennsylvania

- **OR25:** Engineering AAV variants via rational design and directed evolution to escape pre-existing vector immunity
  - Grant Logan, Children’s Medical Research Institute

- **OR26:** Dose-dependent inflammation signatures following Ixo-vec administration in non-human primates
  - Julian Ramos, Adverum Biotechnologies

- **OR27:** Understanding the immune response to adeno-associated virus vectors in the central nervous system
  - Ashley Harkins, University of Massachusetts Chan Medical School

- **OR28:** A redosable non-viral DNA vector platform for highly potent, durable, non-inflammatory gene transfer
  - Jeffrey Bartlett, Rampart Biosciences

#### 08:30-10:30 ∙ Maritime Room, Maison de la Poste ∙ Parallel

**SESSION 3e: Regulatory**

*Chairs: Ilona Reischl, Austrian Medicines and Medical Devices Agency (AGES MEA), Vienna; Patrick Celis, European Medicines Agency, Amsterdam*

- **INV87:** Introduction on the recent activities of CAT – update on the Guideline on investigational ATMPs
  - Ilona Reischl, Austrian Medicines and Medical Devices Agency (AGES MEA), Vienna

- **INV88:** Relevance of New Active Substance (NAS) for ATMPs
  - Patrick Celis, European Medicines Agency, Amsterdam

- **INV89:** Companion diagnostics: what are they and how are they developed
  - Ilona Reischl, Austrian Medicines and Medical Devices Agency (AGES MEA), Vienna

- **INV90:** Experience and use of Real World Data (RWD) in regulatory decision making – Darwin® and Spinal Muscular Atrophy (SMA) registry study
  - Mencia de Lemus Belmonte, CAT patient organisations’ representative, SMA Europe e.V.

- **INV91:** Panel discussion
  - Claire Beuneu, AFMPS, Brussels; Marcos Timón, Agencia espanola de Medicamentos y Productos Sanitarios (AEMPS), Madrid; Alessandro Aiuti, SR-Tiget, Milan; Martina Schüssler-Lenz, Paul-Ehrlich-Institut, Langen

**Panel discussion**

10:30-11:00 ∙ Shed 1

**COFFEE BREAK**

11:00-13:00 ∙ Shed 2A ∙ Plenary

**SESSION 4: Presidential Symposium**

*Chairs: Juan Bueren, CIEMAT/CIBERER/IISS. F. Jiménez Díaz, Madrid; Thierry VandenDriessche, Free University of Brussels*

- **INV24:** Presidential address
  - Juan Bueren, CIEMAT/CIBERER/IISS. F. Jiménez Díaz, Madrid

- **INV25:** Base editing and prime editing: correcting mutations that cause genetic disease in cells, animals, and patients
  - David Liu, Broad Institute, Harvard University, and HHMI - Cambridge, MA

**OUTSTANDING ACHIEVEMENT AWARD**

- Seppo Yla-Herttuala, University of Eastern Finland

**EXCEPTIONAL SERVICE AWARD**

- Hildegard Büning, Hannover Medical School

- Award ceremony for early careers and travel awards
## WEDNESDAY 25 OCTOBER

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<td>13:00-14:30 · Shed 1</td>
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<td>LUNCH</td>
<td>Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University</td>
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<td>13:25-14:25 · Shed 2B</td>
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<td>LUNCHTIME SYMPOSIUM: PTC - Breaking down barriers: Bringing the benefits of gene therapy to patients</td>
<td>Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University</td>
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<td>Chairs: Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University</td>
<td>Welcome, introductions and objectives</td>
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<td>LS01: Rafael Sierra, PTC Therapeutics</td>
<td>PTC Therapeutics: Pioneering for patients – sharing our experience in gene therapy development</td>
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<td>LS02: Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University</td>
<td>From bench to bedside: Getting a gene therapy to patients in need</td>
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<td>LS03: Luca D’Angelo, Sapienza University, Rome</td>
<td>Hands-on treatment experience with an intraputaminal gene therapy</td>
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<td>Q&amp;A</td>
<td>Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University</td>
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<td>13:25-14:25 · Maison de la Poste</td>
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<td>LUNCHTIME SYMPOSIUM: Dyno Therapeutics - NHP-validated capsids for Best-in-Class Ocular and CNS gene delivery</td>
<td>Chair:</td>
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<td>LS04: Eric Kelsic, Dyno Therapeutics</td>
<td>NHP-validated capsids for Best-in-Class Ocular and CNS gene delivery</td>
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<td>13:25-14:25 · BEL</td>
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<td>LUNCHTIME SYMPOSIUM: ERC's funding opportunities: the 2024 Work Programme</td>
<td>Janka Mátrai, ERC</td>
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<td>LS05: Michael Hudecek, Universitätsklinikum Würzburg (UKW)</td>
<td>New targets and technologies for CAR T cells</td>
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<td>LS06: Marcos Timón, Agencia española de Medicamentos y Productos Sanitarios (AEMPS), Madrid</td>
<td>Experience of assessing clinical trials with GMO in Spain</td>
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<td>LS07: Paschalia Koufokotsiou, European Commission, Bruxelles</td>
<td>GMO authorisation for Clinical Trials within the Revision of the EU pharmaceuticals legislation</td>
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### WEDNESDAY 25 OCTOBER

#### SESSION 5a: Infectious diseases & vaccines
Chairs: Anne Galy, ART-TG Inserm, Paris; Michael Muelebach, Paul-Ehrlich-Institut, Langen

**Ine Lentacker**, University of Gent
**INV27**: mRNA Galsomes – making mRNA vaccines more effective, broadly applicable and well-controlled

**ESGCT CAREER PROGRESSION AWARD**
**Alessio Nahmad**, Tabby Therapeutics
**INV28**: Building Better. Engineered B Cells as an Evolving Immunotherapy for Fighting Escape-Prone Diseases

**Kristie Bloom**, Wits/SAMRC Antiviral Gene Therapy Research Unit
**OR29**: Preclinical immunogenicity of synthetic mRNA vaccines for infectious diseases: Mycobacterium Tuberculosis and Hepatitis B virus

**Merve Gülvan**, Institute of Virology, Technical University of Munich/ Helmholtz Centre Munich
**OR30**: Combinatorial knock-down/knock-out strategies to reconstitute antiviral immunity and eliminate persisting hepatitis B virus cccDNA

**Rachel Presti**, Washington University School of Medicine, St. Louis, Missouri
**OR31**: First-in-human trial of systemic CRISPR-Cas9 multiplex gene therapy for functional cure of HIV

**Gabriela Cotugno**, Nouscom Srl
**OR32**: Rapid generation of clinical grade personalized viral vectored vaccines encoding neoantigens for cancer immunotherapy

#### SESSION 5b: Gene therapy for metabolic diseases at clinical stage
Chairs: Nicola Brunetti-Pierri, Tigern, Naples; Rafael Aldabe, CIMA, Pamplona

**Lorenzo D’Antiga**, Azienda Ospedaliera Papa Giovanni XXIII, Bergamo
**INV38**: Liver directed gene therapy: clinical implications

**Maria Escolar**, Forge Biologics
**INV39**: A novel approach for the treatment of inborn errors of metabolism that benefit from HSCT: development of FBX 101 for the treatment of Krabbe disease

**Maria Ester Bernardo**, SR Tiget Milan
**INV91**: Interim skeletal outcome after hematopoietic stem and progenitor cell-gene therapy for Mucopolysaccharidosis type I Hurler

**Ozlem Goker-Alpan**, Lysosomal and Rare Disorders Research and Treatment Center, Fairfax
**OR39**: Results from GALILEO-1, a first-in-human clinical trial of FLT201 gene therapy in patients with Gaucher disease Type 1

**Massayuki Kuroda**, Chiba University
**OR40**: Sustained LCAT replacement in patient with familial LCAT deficiency in clinical trial of ex vivo gene/cell therapy using autologous preadipocytes

#### SESSION 5c: Oncolytic Immunotherapy and Cancer Gene Therapy
Chairs: Vincenzo Cerullo, University of Helsinki, Nicolas Boisgérault, University of Nantes

**Victor van Beusechem**, Amsterdam University Medical Centers
**INV36**: Preclinical and early clinical evaluation of infectivity- and potency-enhanced oncolytic adenoviruses ORCA-010 and ORCA-020.

**Paola Grandi**, CG Oncology
**INV37**: Oncolytic Viral Therapies: Two Knives Against Cancer

**Erwan Sallard**, ZBAF, Witten/Herdecke University
**OR33**: Selection of enhanced oncolytic and gene therapy vectors by Adenovirus Directed EVolution (ADEVO)

**Dmitrii Sorokin**, Exothera
**OR34**: Improving safety and productivity of AdV manufacturing process by versatile adaptation to scale-X manufacturing technology

**Cristian Smerdou**, Cima Universidad de Navarra, IdISNA and CCUN, Pamplona
**OR35**: Self-amplifying RNA vectors encoding interleukin-12 armed with PD-1/PD-L1 blocking nanobodies induce potent antitumor responses

**Shifaa Abdin**, Hannover Medical School
**OR36**: Upscaled production of next generation CAR macrophages derived from human induced pluripotent stem cells for efficient anti-cancer immunotherapy
**WEDNESDAY 25 OCTOBER**

**SESSION 5d: Accessibility of gene therapy**

**Chairs:** Claire Booth, University College London; Johan Prevot, International Patient Organisation for Primary Immunodeficiencies

**Cesar Hernandez,** Spanish Ministry of Health, Madrid  
INV29: Challenges to make possible that patients have timely access to gene therapies: why we need to work together  

**Cornelis Boersma,** Health-Ecore, University Medical Center Groningen, Open University  
INV30: How to fit gene therapies into current healthcare systems?

**Kinnari Patel,** Rocket Pharma  
INV31: Embracing Collaboration and Commitment to Patients to Drive Gene Therapy Forward

**Brian O’Mahony,** Irish Haemophilia Society  
INV32: Accessibility of haemophilia Gene Therapy

**Julio Delgado,** Hospital Clíníc de Barcelona  
INV33: Academic CART-cell development

**Stefano Benvenuti,** Fondazione Telethon  
INV34: Ensuring access to life-saving gene therapy for ultra-rare diseases: a not-for-profit model

**Claire Booth,** University College London  
INV35: Access to effective gene therapies for rare diseases

**George Constandinou,** Thalassaemia International Federation  
INV36: The Bitter Experience - A Thalassaemia Patient’s Perspective.

**Jennifer Adair,**  

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**16:30-17:30 · Shed 2A**

**AGM: ESGCT Annual General Meeting**

ESGCT members are invited to attend our Annual General Meeting to hear updates on the society, our committees and our activities.

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**16:30-19:30 · Gare Maritime**

**COFFEE BREAK | POSTER SESSION I**

17:00-18:15: **UNEVEN** number posters  
18:15-19:30: **EVEN** number posters

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**17:00-18:00 · Maritime, Maison de la Poste**

**EuroGCT Public Engagement Session: Communicating Gene and Cell Therapies to Non-Specialist Audiences**

**Chairs:**

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**19:30 · Gare Maritime**

**MOLECULAR MINGLE**
## Daily Programme

### Thursday 26 October

**08:30-09:00 · Gare Maritime**

**REGISTRATION**

**09:00-10:30 · Shed 2A · PARALLEL**

**SESSION 6a: Hematopoietic diseases gene therapy**

*Chairs:* Giuliana Ferrari, SR-Tiget, Milan; Annarita Miccio, Institut Imagine, Paris

- **INV40:** Daniel Bauer, Harvard Medical School
  - Gene therapy in sickle cell disease: clinical developments and obstacles

- **INV41:** Paula Rio, CIEMAT/CIBERER/III.S. F. Jiménez Díaz, Madrid
  - Improving the efficiency and precision of gene editing in hematopoietic stem cells.

- **OR42:** Luca Biasco, Sana Biotechnology
  - Efficient and specific in vivo genetic engineering of human hematopoietic stem progenitor cells without selective conditioning

**09:00-10:30 · Shed 2B · PARALLEL**

**SESSION 6b: Skin & skeletal diseases**

*Chairs:* Alain Hovnanian, Institut Imagine, Paris; Fernando Larcher, CIEMAT/CIBERER Madrid

- **INV42:** Laura de Rosa, University of Modena and Reggio Emilia
  - Genetically modified epidermal stem cell: a treatment for Epidermolysis Bullosa

- **INV43:** Suma Krishnan, Krystal Bio
  - Rapid and highly efficient transient redosable gene therapy

- **OR43:** Alessandra Zecchillo, SR Tiget, Milan
  - Non-genotoxic conditioning and in vivo gene transfer as new therapeutic approaches for autosomal recessive osteopetrosis

**09:00-10:30 · Maison de la Poste · PARALLEL**

**SESSION 6c: ASGCT**

*Chairs:* Jeffrey Chamberlain, University of Washington

- **INV44:** Jeffrey Chamberlain, University of Washington
  - Delivery and expression of mini- and micro-dystrophins for gene therapy of DMD

- **INV45:** Hans-Pieter Kiem, Fred Hutchinson Cancer Research Center, Seattle
  - In vivo hematopoietic stem cell gene therapy

- **INV46:** Terry Flotte, UMass Chan Medical School
  - Updates and Immune Profiling in AAV gene therapy trials for patients with Tay-Sachs and Sandhoff diseases

**09:00-10:30 · BEL · PARALLEL**

**SESSION 6d: Bleeding disorders**

*Chairs:* Alessio Cantore, SR-Tiget, Milan; Amit Nathwani, University College London

- **INV47:** Thierry VandenDriessche, VUB, Brussels
  - Gene therapy for haemophilia: The end of the beginning?

- **INV48:** Wolfgang Miesbach, Goethe University Hospital in Frankfurt
  - From pivotal study to market approval: important findings for the successful use of gene therapy in haemophilia B

- **OR45:** Bridget Yates, Biomarin Pharmaceutical
  - Exploring actionable strategies to improve AAV5-hFVIII-SQ durability and optimize gene expression

- **OR46:** Gonzalo Martínez Navajas, GENyo, Pfizer-Universidad de Granada-Junta de Andalucía Centre for Genomics and Oncological Research, Granada
  - Lentiviral gene therapy reverts GPIX expression and phenotype in Bernard-Soulier Syndrome type C

**10:30-11:00 · Shed 1**

**COFFEE BREAK**
### THURSDAY 26 OCTOBER

#### SESSION 7: Progress in gene therapies and vaccination: Viral vectors, lipid nanoparticles and mRNAs

**Chairs:** Zoltan Ivics, Paul-Ehrlich-Institut, Langen; Anne Galy, ART-TG Inserm, Paris

- **Klaus Kuehlcie, INV49:** From retroviral vector-based gene therapy to mRNAs for cancer and Covid
- **Ulrike Proetzer, INV50:** Technical University, Munich, Helmholtz Munich
- **Pieter Cullis, INV51:** Development of broadly protective influenza vaccines using nucleoside-modified mRNA

#### LUNCH 13:00-15:00 · Shed 1

**LUNCHTIME SYMPOSIUM: PlasmidFactory - Minicircles: next-generation gene vectors with high potential for clinical applications**

**Chairs:** Martin Schleef, PlasmidFactory GmbH, Bielefeld, Germany; Daniel Scherman, Université Paris Descartes, Paris, France

- **LS08: Martin Schleef, CEO, PlasmidFactory GmbH**
  Welcome address
- **LS09: Michael Hudecek, University Hospital, Würzburg, Germany**
  Advanced strategies for gene transfer and gene editing in CAR-T cells
- **LS10: Kathrin Teschner, Sartorius Xell GmbH, Schloss Holte-Stukenbrock, Germany**
  Use of minicircles for the production of AAV in HEK293 cells
- **LS11: Thijs Gerritzen, Amarna Therapeutics, Leiden, The Netherlands**
  Generation of a new, re-dosable, SV40 viral vector based on minicircle DNA

**Questions and discussion**

#### LUNCHTIME SYMPOSIUM: Orchard Therapeutics 13:30-14:50 · Maison de la Poste

**Chairs:** Juan Bueren, CIEMAT, Madrid; Fulvio Mavilio, Orchard TX, London

- **LS12: Bobby Gaspar, Orchard TX, London**
  Introduction: Orchard’s HSC gene therapy platform
- **LS13: Alessandra Biffi, University of Padua, Padua**
  Gene delivery to the CNS by transplantation of gene-modified HSCs: from neurometabolic to neurodegenerative diseases
- **LS14: Maria Ester Bernardo, HSR-TIGET, Milan**
  Correction of skeletal defects after HSC gene therapy for MPS I (Hurler syndrome)
- **LS15: Pervinder Sagoo, Orchard TX, London**
  Restoring macrophage immune functions by transplantation of gene-modified HSCs: a therapeutic approach to NOD2 Crohn’s disease

**Conclusions**
**THURSDAY 26 OCTOBER**

**LUNCHETIME SYMPOSIUM: HaDEA - The European Health and Digital Executive Agency: ‘European Union support for collaborative research in cell and gene therapy – past, present and future’**

Chair: David Gancberg, Head of Sector - Health innovations and Ecosystems, Unit A3, Health Research, Health and Digital Executive Agency (HaDEA), Belgium

**LS16: David Gancberg, Head of Sector - Unit Health Research, HaDEA, Belgium**
Welcome & overview of EU support for cell and gene therapies, past and present

**LS17: Martin Stoddart, AO-Forschungsinstitut Davos, Switzerland**
Project cmRNAbone: 3D Printed-Matrix Assisted Chemically Modified RNAs Bone Regenerative Therapy for Trauma and Osteoporotic Patients.

**LS18: Annarita Miccio, Institut Imagine, France**
Project EDITSCD: Evaluation of genome-editing approaches for sickle cell disease.

**LS19: Manuel Salmeron-Sanchez, University of Glasgow, UK**
Project HEALIKICK: A modular strategy for the repair of critical sized bone fractures.

**LS20: Cristian Salvador, Fundacion Cidetec, Spain**
Project SINPAIN: A game changer for the treatment of osteoarthritis: a cost effective combined advanced therapy to treat knee osteoarthritis.

**LS21: Dimitrios L. Wagner, Charité - Universitätsmedizin Berlin, Germany**
Project geneTIGA: Gene-edited T cells combating IgA Nephropathy. A blueprint approach for safe & efficient genome editing of T cells to sustainably combat several immune diseases and cancers related to B-cell pathology.

Anna-Pia Papageorgiou, Policy officer, DG Research and Innovation
Panel & audience discussion: What should future funding target in the field through collaborative research?

**SESSION 8a: CART and other engineered T cells**

Chairs: Aude Chapuis, Fred Hutchinson Cancer Center, Seattle; Chiara Bonini, Università Vita-Salute San Raffaele, Milan

**Michael Hudecek, University of Würzburg**
IN53: New targets and technologies for CAR T cells

**Aude Chapuis, Fred Hutchinson Cancer Center, Seattle**
IN54: TCR Therapy: Advancements in Boosting Immune Responses for Cancer Treatment

**Robert Polten, Institute of Experimental Hematology, Hannover Medical School**
OR47: Genetic engineering of natural killer cells for off-the-shelf cell therapy strategies against cervical cancer: targeting Mesothelin and Fibroblast activation protein

**Alessia Potenza, San Raffaele Scientific Institute, Milan**
OR48: Harnessing the adenosine pathway by genome engineering to enhance the functionality of TCR-edited T cell products

**Lorea Jordana, Hemato-Oncology Program, Cima Universidad de Navarra. IdiSNA, Pamplona**
OR49: Identification of key regulatory factors driving CAR-T cell dysfunction in MM by single cell multiomics.

**Alba Rodríguez-Garcia, Institut d’Investigaciones Biomédiques August Pi i Sunyer (IDIBAPS), Barcelona**
OR50: High affinity CAR-T cells exhibit increased resistance to PD-1/PD-L1-mediated inhibition

**SESSION 8b: AAV, non-integrative vectors II**

Chairs: Luk Vandenberghe, Harvard Medical School; Oumeya Adjali, INSERM, Nantes

**Marti Cabanes Creus, Children’s Medical Research Institute, Sydney**
IN55: Not all roads lead to Rome

**Giuseppe Ronzitti, Genethon, Evry**
IN56: Innate immunity to AAV vectors: the devil’s in the details.

**Olena Maiakovska, University of Heidelberg**
OR51: Reconstruction of novel Adeno-associated virus (AAV) genome variants using metagenomic sequencing data

**Hiroki Nakai, Oregon Health & Science University**
OR52: Retrograde renal pelvis injection of select AAV capsids remarkably augments targeted gene delivery to renal tubules with minimum off-target effects

**Robert Nelson, BioAglytix Europe GmbH**
OR53: Evaluation of Cellular Immune Response to Adeno-Associated Virus-Based Gene Therapy

**Rishi Banerjee, University of Helsinki**
OR54: Effective Treatment of Mitochondrial Complex III Deficient Mice with Hepatocyte-Targeted Gene Therapy
### THURSDAY 26 OCTOBER

#### PARALLEL SESSION 8c: Non-viral vectors, nanotechnology & RNA therapeutics

**Chairs:** Enrico Mastrobattista, Utrecht University; Zoltan Ivics, Paul-Ehrlich-Institut, Langen

- **INV57:** Nanoblades allow high-level genome editing in murine and human organoids
  - By Michael Holmes, Tessera therapeutics

- **INV56:** Harnessing mobile genetic elements to write DNA sequences with RNA
  - By Lacramioara Botezatu, Paul-Ehrlich-Institute

- **OR55:** Phenotypic correction of hematopoietic progenitors from Fanconi anemia group A knockout mice using non-viral gene therapy with Sleeping Beauty transposon vectors
  - By Vivian Hegeman, Utrecht University

- **INV58:** OR55: Harnessing mobile genetic elements to write DNA sequences with RNA
  - By Matthew Stanton, Generation Bio

- **OR57:** Phenotypic correction of hematopoietic progenitors from Fanconi anemia group A knockout mice using non-viral gene therapy with Sleeping Beauty transposon vectors
  - By Vivian Hegeman, Utrecht University

#### PARALLEL SESSION 8d: Disease models, IPS cells & organoids

**Chairs:** Vivi Heine, Amsterdam University Medical Center; Oliver Harschnitz, Human Technopole, Milan

- **INV59:** How to engineer human pluripotent stem cells to understand human development and disease
  - By Nuria Montserrat, Catalan Institution for Research and Advanced Studies, Institute for Bioengineering of Catalonia, Barcelona

- **INV60:** Drug discovery of mitochondrial disorders with engineered stem cells and brain organoids
  - By Alessandro Prigione, University of Dusseldorf

- **OR59:** Skeletal muscle organoids for preclinical gene therapy with recombinant AAV vectors
  - By Francesco Saverio Tedesco, University College London

- **OR60:** Human engineered skeletal muscles for advanced modelling of congenital muscular dystrophies and neuromuscular genetic therapies
  - By Mania Ackermann, Fraunhofer Institute for Toxicology and Experimental Medicine (ITEM), Hannover

- **OR61:** Hematopoietic organoids allow for the scalable generation of different immune cell subsets from induced pluripotent stem cells
  - By Deborah Aubin, Stem Cell Medicine Group, Children’s Medical Research Institute, Westmead

- **OR62:** Gene editing for Usher syndrome type 2A: unravelling photoreceptor degeneration mechanisms and exploring therapeutic potential using retinal organoids.
  - By Luigi Naldini, SR Tige, Milan

#### SHED 1

**17:00-17:30:** COFFEE BREAK

#### SHED 2A

**17:30-19:30:** PLENARY SESSION 9: Gene editing

**Chairs:** Julian Grünewald, TUM, Munich; Toni Cathomen, University of Freiburg

- **INV61:** Engineered CRISPR Technologies to Improve Genome Editing
  - By Benjamin Kleinstiver, Mass General Hospital & Harvard Medical School, Boston, MA

- **INV62:** Epitope Engineering for an Immunotherapy “Stealth” Hematopoiesis.
  - By Pietro Genovese, Harvard Medical School, Cambridge, MA

- **INV63:** Advances in In Vivo CRISPR Therapeutics
  - By Laura Sepp Lorenzino, Intelia Therapeutics

- **INV64:** Next Generation Hematopoietic Stem Cell Gene Therapy
  - By Luigi Naldini, SR Tige, Milan

#### GARE MARITIME

**19:30-21:30:** POSTER SESSION II

**19:30-20:30:** EVEN number posters
**20:30-21:30:** UNEVEN number posters
## FRIDAY 27 OCTOBER

### 08:00 - 08:30 · Gare Maritime

**REGISTRATION**

### 08:30 - 10:30 · Shed 2A · PARALLEL

#### SESSION 10a: CNS & sensory diseases II

**Chairs:** Nicole Déglon, CHUV, Lausanne; Nathalie Cartier, AskBio, Paris

- **Richard Porter,** UniQuire
  - INV65: Interim results of AMT-130 (HD-GeneTRX1&2): a gene therapy for Huntington’s Disease

- **Jose Luis Lanciego,** CIMA, University of Navarra, Pamplona
  - INV66: Gene therapy strategies for modeling Parkinson’s disease in non-human primates

- **Pasqualina Coletta,** Stanford University
  - OR63: Therapeutic Efficacy of Brain Repopulation by Hematopoietic-Derived Microglia-Like Cells in Progranulin-Deficient Mice.

- **Yuri Ciervo,** Woman’s and Child Health Department, University of Padua
  - OR64: Development of an ex vivo hematopoietic stem cell gene therapy for frontotemporal dementia (FTD)

- **Marie Anne Burlot,** Coave Therapeutics
  - OR65: Intrastriatal injection of S0112AAV2-GBA1 is an efficient strategy to treat patients suffering from Parkinson’s disease related to GBA1 mutations.

- **Adel Malek,** Tufts Medical Center
  - OR66: A new minimally invasive endovascular approach to the cerebello-pontine cistern enables improved AAV biodistribution in the central nervous system compared to cisterna magna

### 08:30 - 10:30 · Shed 2B · PARALLEL

#### SESSION 10b: Gene editing: Preclinical development

**Chairs:** Angelo Lombardo, SR-Tiget, Milan; Pietro Genovese, Harvard Medical School

- **ESGCT CAREER PROGRESSION AWARD**
  - **Samuele Ferrari,** SR-Tiget, Milan
    - INV67: Uncovering Upsides and Pitfalls of Base and Prime Editing in Hematopoietic Stem Cells
  - **Annarita Miccio,** Institut Imagine, Paris
    - INV68: Genome editing strategies for beta-hemoglobinopathies
  - **Beatriz Olalla,** CIEMAT/CIBERER/IIS, F. Jiménez Díaz, Madrid
    - OR67: Exploring prime editing as a novel approach for targeting diverse Fanconi anemia mutations
  - **Anastasia Conti,** SR TIget, Milan
    - OR68: Inflammation and cellular senescence are uncharted barriers to efficient gene editing of human hematopoietic stem cells and T cells
  - **Manuel Rhiel,** University of Freiburg
    - OR69: Base editors prove non-predictable chromosomal translocations and off-target editing as uncovered by CAST-Seq
  - **Maëlle Ralu,** Genethon, Paris
    - OR70: CRISPR-Cas9 mediated endogenous utrophin upregulation improves Duchenne Muscular Dystrophy

### 08:30 - 10:30 · Maison de la Poste

**PARALLEL 10c: PID gene therapy**

**Chairs:** Marina Cavazzana, Institut Imagine, Paris; Anna Villa, SR-Tiget, Milan

- **Mort Cowan,** UCSF Benioff Children's Hospital
  - INV69: Lentiviral Mediated Gene Therapy for Artemis-Deficient Severe Combined Immunodeficiency

- **Claire Booth,** Great Ormond Street Institute of Child Health, London
  - INV70: Universal Survival and Superior Immune Reconstitution after Lentiviral Gene Therapy with Low Dose Conditioning for X-linked SCID (SCID-X1)

- **Frank Staal,** Leiden University Medical Center
  - OR71: Stem cell based gene therapy for Recombinase deficient-SCID

- **Alessandra Mortellaro,** SR Tiget, Milan
  - OR72: A groundbreaking IL-1RA-based gene therapy platform for treating a spectrum of inflammatory diseases

- **Kerstin Geiger,** University of Freiburg
  - OR73: Base editing restore cellular phenotype of T cells of patients with Hyper-IgE-Syndrome

- **Suk See De Ravin,** National Institute of Health
  - OR74: Base-editing as a safe and highly effective alternative treatment for X-SCID compared to CRISPR-Cas9 nuclease editing with an AAV donor
## Daily Programme

**FRIDAY 27 OCTOBER**

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<th>Time</th>
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<th>Topics</th>
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</table>
| 08:30-10:30 · BEL · PARALLEL | **SESSION 10d: Manufacturing** | | **Eduard Ayuso**, Dinamiqs; **Ulrike Köhl**, Fraunhofer IZI, Leipzig | ** INV71**: Building Flexible End-to-End Cell Therapy Manufacturing Strategies  
**Hanna Lesch**, Exothera, Brussels  
**INV72**: Breaking the myths of challenging large scale AAV manufacturing  
**Nicoletta Loggia**, Orchard Therapeutics  
**INV73**: Meeting the moment: Overcoming manufacturing bottlenecks and technical challenges to usher a new era of genetic medicines  
**James Warren**, Ultragenyx  
**INV74**: Commercial Scale AAV Manufacturing to Deliver Quality Product at High Yield  
**Fabian Jack Hüllen**, RWTH Aachen University  
**OR75**: Establishment of a genome-wide CRISPR/Cas9 screening method for the identification of cellular factors that affect AAV production  
**Pedro Vicente**, IBET/ITQB-NOVA  
**OR76**: Advancing the manufacture of hiPSC-derived cardiomyocytes in bioreactors through Wnt activation and dissolved oxygen oxygen control |
| 10:30-11:00 · Shed 1 | **COFFEE BREAK** | | | |
| 11:00-13:00 · Shed 2A · PARALLEL | **SESSION 11a: Gene editing: Towards clinical trials** | | **Alessandro Aiuti**, SR-Tiget, Milan; **Laura Sepp Lorenzino**, Intelia Therapeutics | **INV75**: Early phase trials of crispr/cas9 and base edited CAR T cells  
**Franco Locatelli**, Catholic University of the Sacred Heart, RCCS, Bambino Gesù Hospital, Rome  
**INV76**: Gene editing in patients with hemoglobinopathies  
**Richard Morgan**, Be Biopharma  
**OR77**: Development of an ex vivo precision gene engineered B cell medicine platform and demonstration of engraftment without pre-conditioning in non-human primates  
**Alissa Müller**, Institute of Molecular and Clinical Ophthalmology Basel  
**INV78**: Adenine-base editing corrects the most common ABCA4 mutation causing Stargardt disease  
**Jonathon Winnay**, Prime Medicine  
**OR79**: Prime editing precisely corrects prevalent pathogenic mutations observed in Glycogen Storage Disease Type 1b (GSD1b) patients  
**OR80**: EMD-301 - a potent “one and done” gene editing-based therapy for hypercholesterolemia-related disorders up regulates LDLR expression and boosts LDL-C uptake |
| 11:00-13:00 · Shed 2B · PARALLEL | **SESSION 11b: CNS & sensory diseases III** | | **Alberto Auricchio**, Tigem, Naples; **Ellen Reisinger**, University of Tübingen | **INV77**: Considerations in the development of a gene therapy for Otoferlin gene-mediated hearing loss  
**Amy Geard**, UCL London  
**OR81**: AAV9-Mediated Gene Therapy In A Knock-In Mouse Model Of Infantile Neuroaxonal Dystrophy  
**Barbara Nguyen-vu**, MeiraGTx  
**OR82**: Gene therapy for ALS and FTD: Preclinical efficacy of AAV-HUPF1 optimized for clinical translation with improved vector genome and novel CNS capsid.  
**Sergi Verdes**, Universitat Autònoma de Barcelona (UAB), Bellaterra  
**OR83**: Promoting functional improvement in ALS through myotrophic AAV-mediated overexpression of α-Klotho  
**Eva Andres-Mateos**, Atensa Therapeutics  
**OR84**: IND-enabling studies to support the clinical development of ATSN-201, a subretinally delivered, laterally spreading gene replacement therapy for X-Linked Retinoschisis (XLRIS)  
**Yong Tao**, Shanghai Ninth People’s Hospital, Shanghai Jiao Tong University School of Medicine, Shanghai  
**OR85**: A single AAV packed with RNA base editor treats OTOF-mediated deafness  
**Yilai Shu**, ENT institute and Otorhinolaryngology Department of Eye & ENT Hospital, State Key Laboratory of Medical Neurobiology and MOE Frontiers Center for Brain Science, Fudan University, Shanghai, China  
**OR86**: AAV1-hOTOF gene therapy trial for autosomal recessive deafness 9 (DFNB9) |
## FRIDAY 27 OCTOBER

### SESSION 11c: Update on immune responses to gene & cell therapy

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<tr>
<td>11:00-13:00</td>
<td>Maison de la Poste</td>
<td>PARALLEL</td>
<td>Sylvain Fisson, Genethon, Evry; Oumeya Adjali, INSERM UMR 1089, Nantes</td>
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<td><strong>INV78:</strong> HLA-dependent risk of T cell response to micro-dystrophin transgene in DMD patients harboring mutations in dystrophin N-terminal exons</td>
<td>Helene Haegel, Roche</td>
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<td><strong>INV79:</strong> Addressing AAV vector immunogenicity in humans</td>
<td>Federico Mingozzi, Spark Therapeutics</td>
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<td><strong>INV80:</strong> HLA-dependent risk of T cell response to micro-dystrophin transgene in DMD patients harboring mutations in dystrophin N-terminal exons</td>
<td>Gwladys Gernoux, Université de Nantes, CHU de Nantes, INSERM, TaRGeT - Translational Research in Gene Therapy, UMR 1089</td>
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<td><strong>INV81:</strong> Addressing AAV vector immunogenicity in humans</td>
<td>OR87: Anti-CD45SRC antibody leads to hepatocyte preservation after systemic AAV gene transfer in a humanized mouse model mimicking pre-existing anti-AAV T cell response</td>
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<td><strong>INV82:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Maddalena Migliavacca, SR Tigon, Milan</td>
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<td><strong>INV83:</strong> HLA-dependent risk of T cell response to micro-dystrophin transgene in DMD patients harboring mutations in dystrophin N-terminal exons</td>
<td>OR88: Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
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<td><strong>INV84:</strong> Addressing AAV vector immunogenicity in humans</td>
<td>OR89: Immune modulation and the role of genetic diversity in translational animal models of AAV gene therapy</td>
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### SESSION 11d: Cardiovascular & muscular diseases

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<td>11:00-13:00</td>
<td>Bel</td>
<td>PARALLEL</td>
<td>Mauro Giacca, Kings College London, Seppo Yla-Herttuala, University of Eastern Finland, Kuopio</td>
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<td><strong>INV85:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Christian Kupatt, Technical University Munich</td>
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<td><strong>INV86:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Jonathan Schwartz, Rocket Pharma</td>
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<td><strong>INV87:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Nicolai Preisler, Rigshospitalet, Copenhagen</td>
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<td><strong>INV88:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Hichem Tasfou, University of Washington</td>
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<td><strong>INV89:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Ronald Crystal, Weill Cornell Medical College</td>
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<td><strong>INV90:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Debanjana Majumdar, VUB Brussels</td>
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<td><strong>INV91:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Debajana Majumdar, VUB Brussels</td>
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<td><strong>INV92:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Debajana Majumdar, VUB Brussels</td>
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<td><strong>INV93:</strong> Gene therapy For Adenosine Deaminase Deficiency: Post-marketing Experience and Long-term Outcome</td>
<td>Debajana Majumdar, VUB Brussels</td>
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<td>13:00-14:30</td>
<td>Shed 1</td>
<td>Lunch</td>
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### LUNCHTIME SYMPOSIUM: MaxCyte - Next-generation CAR-based immunotherapies

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<tr>
<td>13:25-14:25</td>
<td>Shed 2B</td>
<td>LUNCHTIME SYMPOSIUM: MaxCyte - Next-generation CAR-based immunotherapies</td>
<td>Lesley Eschinger, MaxCyte</td>
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<td><strong>LS22:</strong> Michael Hudecek, University of Würzburg. Next-generation CAR-based immunotherapies</td>
<td>Lesley Eschinger, MaxCyte</td>
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### LUNCHTIME SYMPOSIUM: EIC Cell and Gene Therapy (CGT) community

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<td>13:25-14:25</td>
<td>Bel</td>
<td>LUNCHTIME SYMPOSIUM: EIC Cell and Gene Therapy (CGT) community</td>
<td>Iordanis Arzimanoglou, EIC Programme Manager for Health &amp; Biotech</td>
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<td><strong>LS23:</strong> Hans-Peter Klem, Fred Hutchinson Cancer Center, Seattle Prospects for in vivo hematopoietic stem cell gene therapy</td>
<td>Anna Panagopoulou, ERA &amp; Innovation, DG R&amp;I &amp; Stéphane Ouaki, EIC Welcome</td>
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<td><strong>LS24:</strong> Michael Wenger, BioNTech</td>
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<td>BioNTech’s oncology pipeline with focus on mRNA moieties and cell and gene therapies</td>
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<td><strong>LS25:</strong> Seppo Yla-Herttuala, Biocenter Kuopio, EU EATRIS-ERIC and National Virus Vector Laboratory</td>
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<td>EIC portfolio, an opportunity for EU science and innovative industry</td>
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<td><strong>Panel discussion:</strong> John Hodgson, former Editor-at-Large, Nature Biotechnology</td>
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<td><strong>Panel discussion:</strong> John Hodgson, former Editor-at-Large, Nature Biotechnology</td>
<td>Mariana Werner Sunderland, Achilles Therapeutics, Ltd., UK, Bernhard Gentner, Ludwig Institute for Cancer Research Lausanne</td>
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<td><strong>Panel discussion:</strong> John Hodgson, former Editor-at-Large, Nature Biotechnology</td>
<td>Luc Henry, Limula, Luca Gattinoni, Leibniz Institute for Immunotherapy, Alberto Auricchio, Tigem, Naples</td>
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<td>14:30-17:00</td>
<td>Shed 2A</td>
<td>SESSION 12: From Advanced Translational Studies to Approved Therapies</td>
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<td>Chairs: Juan Bueren, CIEMAT/CIBERER/IIS, F. Jiménez Diaz, Madrid; Thierry VandenDriessche, VUB Brussels</td>
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<td>Mauro Giacca, Kings College London</td>
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<td>INV82: RNA therapies for cardiac regeneration and precise gene editing</td>
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<td>Tomoki Todo, The University of Tokyo</td>
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<td>INV83: Development and approval of oncolytic herpes virus G47Δ for malignant glioma in Japan</td>
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<td>Paul Wuh-Liang Hwu, National Taiwan University Hospital and China Medical University Hospital</td>
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<td>INV84: Long term clinical trials for eladocagene exuparvovec, a gene therapy for AADC deficiency</td>
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<td>Alessandro Aiuti, SR Tiget, Milan</td>
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<td>INV85: Gene therapy for ADA-SCID: from clinical trial to market experience and the future of rare disease gene therapy</td>
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