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12.00-12.15	OR030 Chemically modified guide RNAs enhance CRISPR/Cas genome editing in human primary cells *Rasmus Bak, Stanford University, CA*
12.15-12.30	Questions
10.30-12.30 *Helsinki Hall*	**3b: Neural diseases** *Chairs: Nathalie Cartier-Lacave, Karen Kozarsky*
10.30-11.00	INV055 Development of a neuronal gene therapy approach for Friedreich Ataxia *Françoise Piguet, INSERM U596; CNRS, UMR7104; Université de Strasbourg*
11.00-11.30	INV056 Hematopoietic stem cell based gene therapy for the treatment of lysosomal storage disorders *Alessandra Biffi, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
	Proffered papers
11.30-11.45	OR031 Intra-cerebral administration of AAV vector containing the human alpha-N-acetylglucosaminidase cDNA in children with Sanfilippo type B (MPSIIIB) syndrome: results at 12 months of a phase I/II trial *Marc Tardieu, Université Paris-Sud*
11.45-12.00	OR032 Patient-specific gene-corrected iPSC-derived neural stem/progenitor cells for autologous cell therapy applications in lysosomal storage diseases *Angela Gritti, San Raffaele Scientific Institute, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
12.00-12.15	OR033 AAV- APPsα brain delivery rescues synaptic failure in an Alzheimer's disease mouse model *Romain Fol, INSERM U1169 / MIRCen CEA Fontenay aux Roses, Université Paris-Sud; Université Paris Descartes*
12.15-12.30	Questions
10.30-12.30 *Veranda 1*	**3c: Blood-related diseases** *Chairs: Juan Bueren, Sandeep Soni*
10.30-11.00	INV057 Gene therapy for primary immunodeficiencies in Japan *Masafumi Onodera, National Center for Child Health and Development, Tokyo*
11.00-11.30	INV058 Gene therapy for beta-thalassemia: moving from the preclinical phase to the clinical trial *Giuliana Ferrari, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*

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	Proffered papers
11.30-11.45	OR034 Lentiviral-mediated correction of mobilized CD34+ progenitors and repopulating cells from Fanconi anemia patients *Juan Bueren, CIEMAT/CIBERER, Madrid*
11.45-12.00	OR035 A new conditional mouse model to unravel the platelet defect in Wiskott–Aldrich syndrome *Lucia Sereni, Vita-Salute San Raffaele University; HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
12.00-12.15	OR036 A prospective clinical trial of autologous TARGT™ prolonged EPO secretion showed EPO-independence in EPO-dependent ESRD patients *Shany Blum, Medgenics Medical Israel, Ltd, Misgav*
12.15-12.30	OR037 Correction of CTLs cytotoxic function defect by SIN-lentiviral mediated expression of Munc13-4 in type 3 familial hemophagocytic lymphohistiocytosis *Tayebeh Soheili, Institut Imagine, Inserm U1163, Paris*
10.30-12.30 *Veranda 2*	**3d: Muscle and mesenchyme-related diseases** *Chairs: Thierry vandenDriessche, Nicholas Mazarakis*
10.30-11.00	INV059 Gene therapy for inherited muscle diseases *Fulvio Mavilio, Généthon, Evry*
	Proffered papers
11.00-11.15	OR038 Next-generation muscle-directed gene therapy using skeletal-muscle specific transcriptional modules identified by genome-wide computational analysis *Marinee K L Chuah, Free University of Brussels; KU Leuven*
11.15-11.30	OR039 Neuroprotection in an ALS mouse model following peripheral delivery of motor neuron targeted aCAR-IGF-1 Lentiviral vector *Nicholas Mazarakis, Imperial College London*
11.30-11.45	OR040 Spell-checking nature: interrogating the versatility of the CRISPR/Cas9 system for the treatment of Duchenne Muscular Dystrophy *Daria Wojtal, Hospital for Sick Children; University of Toronto*
11.45-12.00	OR041 Adenoviral vector transduction of designer nucleases restores DMD reading frames in dystrophin-defective muscle cell populations *Ignazio Maggio, Leiden University Medical Center*
12.00-12.15	OR042 Human artificial chromosome-mediated genetic correction of human dystrophic skeletal muscle progenitors for the autologous cell therapy of Duchenne muscular dystrophy *Francesco Saverio Tedesco, University College London*

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12.15-12.30	OR043 Gene transfer of MTMR2 prolongs survival and rescues the pathology in a murine model of myotubular myopathy *Ana Buj Bello, Généthon, Evry*
12.30-13.30 *Piazza*	Lunch
13.30-14.30 *Finlandia Hall*	**4: Genetic medicine: current challenges and future prospects** *Chairs: Seppo Ylä-Herttuala, Michel Sadelain*
13.30-14.00	INV060 Defining, modifying, and improving the specificities of CRISPR/Cas9 nucleases *Keith Joung, Massachusetts General Hospital; Harvard Medical School*
14.00-14.30	INV061 DNA integration in human gene therapy *Frederic Bushman, University of Pennsylvania*
	Parallel sessions 4a, 4b, 4c
14.30-16.15 *Finlandia Hall*	**4a: Metabolic diseases and primary immune deficiencies** *Chairs: Alessandro Aiuti, Jenni Huusko*
14.30-15.00	INV062 Evolving gene therapy for primary immunodeficiency *Adrian Thrasher, University College London*
	Proffered papers
15.00-15.15	OR044 Pre-clinical workup of lentiviral mediated stem cell gene therapy for mucopolysaccharidosis type IIIA *Brian Bigger, University of Manchester*
15.15-15.30	OR045 Efficient targeted gene addition to a safe harbor locus in long-term repopulating hematopoietic stem cells for correction of X-linked Chronic Granulomatous Disease via genome editing *Fyodor Urnov, Sangamo BioSciences Inc, Richmond CA*
15.30-15.45	OR046 *Ex vivo* liver-directed gene therapy in a pig model of hereditary tyrosinemia type 1 *Raymond Hickey, Mayo Clinic, Rochester MN*
15.45-16.00	OR047 Guanylyl cyclase A-targeted gene therapy for polycystic kidney disease *Yasuhiro Ikeda, Mayo Clinic, Rochester MN*
16.00-16.15	OR048 Human T effector and T regulatory cell differentiation and response are distinctly controlled by FOXP3 expression *Francesca Santoni de Sio, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*

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14.30-16.15 *Helsinki Hall*	**4b: Oncolytic vectors and therapies** *Chairs: Vincenzo Cerullo, Hrvoje Miletic*
14.30-15.00	INV065 Systemic administration of VSV expressing cDNA libraries to treat established tumours *Richard Vile*
15.00-15.30	INV066 Oncolytic adenoviruses: versative tools for killing tumor cells, inducing anti-tumor immunity and facilitating T-cell therapy. A summary of rodent and human data *Akseli Hemminki, Haartman Institute, University of Helsinki; Helsinki University Hospital Comprehensive Cancer Center; TILT Biotherapeutics Ltd*
	Proffered papers
15.30-15.45	OR052 Development of oncolytic vaccine for cancer treatment *Vincenzo Cerullo, Faculty of Pharmacy, University of Helsinki*
15.45-16.00	OR053 An oncolytic adenovirus (AdΔ19K) sensitises pancreatic cancer cells to cytotoxic drugs by promoting DNA-damage and aberrant mitosis through inactivation of checkpoint mediators *Gunnel Halldén, Barts Cancer Institute, Queen Mary University of London*
16.00-16.15	OR054 CD30-targeted oncolytic virotherapy of Hodgkin Lymphoma *Alexander Muik, Paul Ehrlich Institute, Langen*
14.30-16.15 *Veranda 1*	**4c: Non-viral vectors** *Chairs: Samir El-Andaloussi, Zoltan Ivics*
14.30-15.00	INV063 Oligonucleotides for splice-switching and DNA duplex invasion *C. I. Edvard Smith, Karolinska Institutet, Karolinska University Hospital Huddinge, Stockholm*
15.00-15.30	INV064 The TargetAMD project – Using Free of Antibiotic Resistance gene (pFAR4) miniplasmids for a Sleeping Beauty (SB100X) mediated gene therapy to treat neovascular Age-Related Macular Degeneration (nAMD) *Gabriele Thumann, Department of Ophthalmology, University Hospitals of Geneva*
	Proffered papers
15.30-15.45	OR049 A Phase 2b clinical trial of non-viral gene therapy in Cystic Fibrosis patients: randomised, double-blind, placebo-controlled repeated aerosol delivery to the lungs *Deborah Gil, UK Cystic Fibrosis Gene Therapy Consortium*

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15.45-16.00	OR050 Efficient non-viral gene delivery by minicircle Sleeping Beauty transposon system into hematopoietic stem cells for gene therapy applications *Marta Holstein, Paul Ehrlich Institute, Langen*
16.00-16.15	OR051 Ultrasound-targeted delivery of chemotherapeutic drug and nucleic acids by gas-filled cationic liposomes *Anthony Delalande, CNRS, Université d'Orléans*
16.15-16.45 *Piazza*	Afternoon break
	Parallel sessions 5a, 5b, 5c, 5d
16.45-18.45 *Finlandia Hall*	**5a: Modelling and IPS cells** *Chairs: Juan Bueren, Matti Korhonen*
16.45-17.15	INV067 rAAV-mediated gene therapy for metabolic liver disease; prospects and challenges in paediatric patients *Ian Alexander, Children's Hospital at Westmead; Children's Medical Research Institute; University of Sydney*
17.15-17.45	INV068 AAV-based delivery of APP and PS1 in adult mouse hippocampus identifies initial steps of Alzheimer's disease *Nathalie Cartier-Lacave, INSERM UMR1169, Université Paris-Sud; CEA, DSV, FBM, MIRCen, Fontenay-aux-Roses*
17.45-18.15	INV069 Pluripotent stem cell (PSC)-derived myeloid cells as a novel source for cell and gene therapy strategies *Tom Moritz, REBIRTH Cluster of Excellence, Hannover Medical School*
	Proffered papers
18.15-18.30	OR055 Pulmonary Macrophage Transplantation employing HSC- or iPSC-derived cells as an innovative gene therapy approach in Pulmonary Alveolar Proteinosis *Miriam Hetzel, Hannover Medical School*
18.30-18.45	OR056 Comprehensive atlas of activity and "integrome" of human hematopoietic stem/progenitor cells *Serena Scala, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
16.45-18.45 *Helsinki Hall*	**5b: Exosomes and nanoparticles** *Chairs: C. I. Edvard Smith, Petri Mäkinen*
16.45-17.15	INV070 Engineered extracellular vesicles for biomedical applications *Samir El-Andaloussi, Karolinska Institutet; University of Oxford*

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17.15-17.45	INV071 G-force loading of virus vectors into vesicles for enhanced gene therapy vehicles *Zachary Fitzpatrick, Massachusetts General Hospital*
	Proffered papers
17.45-18.00	OR057 The use of transgenic extracellular vesicles (EVs) expressing membrane-bound CD39/CD73 for treatment of inflammatory disease *Susanne Snoek, Academic Medical Center; Arthrogen BV*
18.00-18.15	OR058 Extracellular membrane vesicles from umbilical cord blood derived mesenchymal stromal cells protect against ischemic acute kidney injury, a feature that is lost after inflammatory conditioning *Lotta Kilpinen, Finnish Red Cross Blood Service, Helsinki*
18.15-18.30	OR059 Polyplex nanomicelles assembled with neprilysin mRNA augmented clearance of beta-amyloid peptide from intracerebroventricular infusion *Chin-Yu Lin, University of Tokyo*
18.30-18.45	OR060 Exosomes from human Cardiac Progenitor Cells: development of GMP-grade manufacturing and testing methods *Marina Radrizzani, Cardiocentro Ticino*
16.45-18.45 *Veranda 1*	**5c: Regulatory issues** *Chairs: Minna Hassinen, Otto Merten* bluebirdbio
16.45-17.15	INV072 Latest news from the CAT – progresses and challenges in development of ATMPs *Paula Salmikangas, Finnish Medicines Agency*
17.15-17.45	INV073 Accelerating the development of gene therapy products: a global regulatory view *Anne Virginie Eggiman, bluebird bio, Cambridge MA*
17.45-18.15	INV074 Obstacles and opportunities to gene therapy approval and usage for clinical disease *Yuman Fong, City of Hope, Duarte CA*
	Proffered papers
18.15-18.30	OR061 Does the common use of national regulatory pathways undermine the general European regulation on Advanced Therapy Medicinal Products? *Aurélie Mahalatchimy, University of Sussex*
18.30-18.45	OR062 Environmental risk assessment for gene and cell therapy products: tips and tricks to avoid pitfalls with clinical trial and market authorisation applications *Ursula Jenal, Jenal & Partners Biosafety Consulting, Rheinfelden*

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16.45-18.45 *Veranda 2*	**5d: Vascular biology and imaging** *Chairs: Johanna Laakkonen, Roger Hajjar*
16.45-17.15	INV075 Translational insights into vascular growth factors *Kari Alitalo, Biomedicum Helsinki, University of Helsinki*
17.15-17.45	INV076 Cardiovascular molecular imaging in health and disease *Juhani Knuuti, University of Turku; Turku University Hospital*
17.45-18.15	INV077 Gene therapy for heart and lung transplantation *Antti Nykänen, University of Helsinki*
	Proffered papers
18.15-18.30	OR063 Vammin induces a highly efficient angiogenic response through VEGFR-2/NRP-1 and bypasses the regulatory function of VEGFR-1 *Pyry Toivanen, A.I. Virtanen Institute, University of Eastern Finland, Kuopio*
18.30-18.45	OR064 EphrinB2/EphB4 signaling controls the switch between normal and aberrant angiogenesis by VEGF *Andrea Banfi, University of Basel; Basel University Hospital*
20.00-01.00	Molecular Mingle gala evening – Restaurant Sipuli. See page 44 *Carriages will depart from Finlandia Hall at 19.30. Return coaches will depart at 23.00 and midnight*