PROGRAMME

THURSDAY 23 OCTOBER 2014

MAIN CONGRESS	
17.00	**1: Highlights of clinical progress**
Auditorium	*Chairs: Luigi Naldini, Fulvio Mavilio* Opening and welcome *Gerard Wagemaker, Erasmus Medical Center, Rotterdam* INV032 Hematopoietic stem cell gene therapy for lysosomal storage disorders *Alessandra Biffi, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* INV033 Immunological and metabolic correction after lentiviral vector mediated hematopoietic stem cell gene therapy for ADA deficiency *Bobby Gaspar, UCL Institute of Child Health, London* INV034 Clinical progress update *Marina Cavazzana, Hôpital Universitaire Necker – Enfants Malades, Paris* INV035 Gene therapy for Wiskott-Aldrich Syndrome *Alessandro Aiuti, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
18.30	**Welcome reception – Poster Session 1** *Onyx room* Posters 1–103. See page 33 for details

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MAIN CONGRESS	
08.30	**2: Highlights of clinical progress II**
Auditorium	*Chairs: David Kirn, Len Seymour* INV036 Targeted oncolytic and immunotherapeutic viruses: emerging multi-mechanistic biologics for cancer *David Kirn, 4D Molecular Therapeutics; SillaJen, San Francisco, CA* INV037 Enadenotucirev, a group B oncolytic adenovirus: assessment of potency, safety and selectivity *Kerry Fisher, PsiOxus Therapeutics, Oxford* INV038 What T-cells see on human cancer *Ton Schumacher, The Netherlands Cancer Institute, Amsterdam*
10.00	Morning break
10.30	**Parallel sessions 2a, 2b, 2c, 2d**
Auditorium	**2a: AAV Vectors** *Chairs: Hildegard Büning, David Schaffer* INV039 Directed evolution of new adeno-associated viruses for therapeutic gene delivery *David Schaffer, 4D Molecular Therapeutics, San Francisco, CA; University of California, Berkeley* INV040 Pre-existing immunity to AAV overcome by in silico ancestral capsid design *Luk Vandenberghe, Harvard Medical School, Boston, MA* *Proffered papers* OR084 Off-target-free gene delivery to clinically relevant cell types by receptor-targeted adeno-associated viral vectors *Robert Münch, Paul-Ehrlich-Institut, Langen* OR085 Capsid-engineering overcomes barriers toward endothelial cell transduction *Li-Ang Zhang, University of Cologne* OR086 Successful repeated hepatic gene delivery in mice and non-human primates achieved by sequential administration of AAV2/5 and AAV1 vector serotypes *Anna Majowicz, uniQure BV, Amsterdam; CIMA, University of Navarra, Pamplona* OR087 Exhaustive characterisation of DNA contaminants in rAAV productions by next generation sequencing *Benjamin Cogné, University of Nantes; INSERM, UMR 1089, Nantes*

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10.30 *Mississippi*	**2b: Innate immunity and stem cell therapy** *Chairs: Federico Mingozzi, Axel Schambach, Thomas Chalberg* INV041 The great escape: immune evasion by Epstein-Barr virus *Maaike Ressing, Leiden University Medical Centre* INV042 Adeno-associated viral (AAV) vectors are the most promising platform for *in vivo* gene transfer *Federico Mingozzi, Généthon, Evry* *Proffered papers* OR005 Stimulated granulocyte differentiation in an induced pluripotent stem cell model of severe congenital neutropenia by vitamin B3 *Dirk Hoffmann, Hannover Medical School* OR006 Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatous disease *Giada Farinelli, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR007 Ectopic gp91phox expression is detrimental to XCGD iPS cell-derived neutrophils *Huan-Ting Lin, University of Tokyo* OR008 Large-scale hematopoietic differentiation of human induced pluripotent stem cells provides granulocytes or macrophages for cell and gene therapies *Thomas Moritz, REBIRTH Cluster of Excellence, Hannover Medical School*
10.30 *Amazon*	**2c: Muscle gene and cell therapy** *Chairs: Hidde Haisma, Johnny Huard* INV043 The role of stem cell exhaustion in aging and disease *Johnny Huard, University of Pittsburgh, PA* INV044 Gene replacement therapy for myotubular myopathy *Anna Buj-Bello, Généthon, Evry* *Proffered papers* OR009 Adeno-associated virus vector (AAV) microdystrophin gene therapy for Duchenne muscular dystrophy OR011 Dystrophin deficient rats: generation and characterisation of a new model for Duchenne Muscular Dystrophy *Caroline Le Guiner, Généthon, Evry* OR010 MMP-9 serum levels increase over time in Duchenne Muscular Dystrophy patients and decrease upon treatment with drisapersen *Pietro Spitali, Leiden University Medical Centre*

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10.30 *Yangtze*	**2d: Cardiovascular and pulmonary diseases** *Chairs: Seppo Ylä-Herttuala, Sarah Ferber* INV045 Cardiac regeneration: Stem cells and beyond *Marie Jose Goumans, Leiden University Medical Centre* INV046 Systemic delivery of AAVrh10 expressing frataxin corrects the severe mitochondrial cardiomyopathy of frataxin deficient mice *Hélène Puccio, IGBMC, Strasbourg* *Proffered papers* OR012 Molecular mechanisms of vascular hyperpermeability in VEGF therapy *Johanna Laakkonen, University of Eastern Finland, Kuopio* OR013 'First in human' clinical trial using allogeneic Cardiac Stem/Progenitor Cell (CSCs) for Acute Myocardial Infarction (AMI) treatment. *Luis Rodriguez-Borlado, Coretherapix S L, Madrid* OR014 A PET based approach for highly sensitive monitoring of teratoma formation from pluripotent stem cells *Cajetan Lang, University of Rostock* OR015 Pulmonary transplantation of multipotent or pluripotent-stem cell derived macrophage progenitors as a novel treatment option for Pulmonary Alveolar Proteinosis *Nico Lachmann, REBIRTH Cluster-of Excellence, Hannover Medical School*
12.30	ESGCT General Assembly – Auditorium
12.30	Lunch – Exhibition and posters
14.30	**3: Emerging technologies in hematopoietic stem cell gene therapy**
Auditorium	*Chairs: Luigi Naldini, Keith Joung* INV047 Targeted genome and epigenome editing using engineered CRISPR-Cas and TALE technologies *Keith Joung, Harvard Medical School, Boston, MA* INV048 Development and applications of CRISPR-Cas9 for genome editing *Feng Zhang, The Broad Institute, Cambridge, MA* INV049 Prosthetic networks – synthetic biology-inspired treatment strategies for metabolic disorders *Martin Fussenegger, Swiss Federal Institute of Technology, Zürich*
16.00	Afternoon break

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16.30	**Parallel Sessions 3a, 3b, 3c, 3d**
Auditorium	**3a: Genetic vaccines and Ad vectors** *Chairs: Kerry Fisher, Dale Vanderputten* INV050 The use of AAV vectors to express antibodies to prevent and/or treat infectious diseases *James Wilson, University of Pennsylvania* INV051 *In vivo* barriers and advances in adenovirus vector technology *Stefan Kochaneck, University of Ulm* *Proffered papers* OR016 Oncolytic adenovirus loaded with MHC-I restricted peptide as platform for oncolytic vaccine *Vincenzo Cerullo, University of Helsinki* OR017 Lentiviral-based anti-HIV therapeutic vaccine: design, preclinical studies and phaseI/II clinical trial preliminary results *Cécile Bauche, Theravectys, Villejuif* OR018 Development of a next generation Semliki Forest virus-based DNA vaccine against cervical cancer *Stephanie van de Wall, University of Groningen* OR019 ORCA-010, a novel potency enhanced oncolytic adenovirus, exerts strong antitumor activity in preclinical models *Wenliang Dong, ORCA Therapeutics BV; VU University, Amsterdam*
16.30 *Mississippi*	**3b: Epigenetic regulation and gene editing I** *Chairs: Didier Trono, Hidde Haisma* INV052 Architecture and dynamics of genome-nuclear lamina interactions *Bas van Steensel, Netherlands Cancer Institute, Amsterdam* INV053 Transposable elements and their epigenetic control mechanisms are key regulators of transcriptional networks in pluripotent stem cells *Didier Trono, EPFL, Lausanne* *Proffered papers* OR020 Targeted genome editing in human long-term repopulating hematopoietic stem cells for correction of SCID-X1 *Pietro Genovese, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR021 Targeted genome editing by lentiviral protein transduction of ZFN and Cas9 proteins *Yujia Cai, Aarhus University*

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	OR022 CCR5-Uco-TALEN: A novel highly active TAL effector nuclease (TALEN) for the targeted knock-out of the HIV-co-receptor CCR5 *Ulrike Mock, University Medical Centre Hamburg-Eppendorf* OR023 Towards TALEN-mediated targeting of FANCA in the AAVS1-homolog safe harbor locus in FA-A mice *Maria Jose Pino-Barrio, CIEMAT/CIBERER, Madrid*
16.30 *Amazon*	**3c: Gene and cell therapy for ocular diseases** *Chairs: Robin Ali, Kyriacos Mitrophanous* INV054 WNT7A and PAX6 define corneal epithelium homeostasis and pathogenesis *Kang Zhang, University of California, San Diego* INV055 Engineering light responses and outer segments *Botond Roska, Friedrich Miescher Institute for Biomedical Research, Basel* *Proffered papers* OR024 Evaluation of an optimised injection system for retinal gene therapy *Dominik Fischer, University Hospital, Tübingen; University of Oxford* OR025 Preventing visual loss after corneal trauma by targeting miR-145 *Christiane Gras, Hannover Medical School* OR026 Effective delivery of large genes to the retina by dual AAV vectors *Ivana Trapani, TIGEM, Naples* OR027 Development of EncorStat®, donor corneal tissue genetically engineered to prevent graft rejection *Scott Ellis, Oxford BioMedica Ltd*
16.30 *Yangtze*	**3d: Genetic instability syndromes and vector genotoxicity** *Chairs: Juan Bueren, Christof von Kalle* INV056 *Mutatis mutandis*: why a cell makes mutations and how this can benefit our fitness *Niels De Wind, Leiden University Medical Centre* INV057 Towards the lentiviral gene therapy of mobilised CD34+ cells from Fanconi anemia patients *Juan Bueren, CIEMAT/CIBERER, Madrid* *Proffered papers* OR028 Comparative AAV wild-type and rAAV vector-mediated genomic integration profiles in human diploid fibroblasts analysed by 3rd generation PacBio DNA sequencing *Regine Heilbronn, Charité University Medicine, Berlin*

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	OR029 A new bioinformatics tool to improve precision and quality of vector integration site identification after genomic alignment *Andrea Calabria, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR030 High throughput, high nucleotide resolution integration site analysis of wtAAV reveals viral instability during the process of integration *Karl Petri, National Center for Tumor Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg* OR031 Targeted Sequencing for Detection of Vector Integration Sites *Stefan Wilkening, National Center for Tumor Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg*
18.30	**Poster session 2 – Onyx room** Posters 104–197. See page 33 for details
20.00	Speakers' Dinner – by invitation only *Coaches will depart from the World Forum main entrance at 19.45*

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MAIN CONGRESS	
08.30	**4: Gene and cell therapy for cancer**
Auditorium	*Chairs: Rob Hoeben, Victor van Beusechem* INV058 TRUCKs: the new generation of CARs *Hinrich Abken, University of Cologne* INV059 Development of Chimeric Antigens Receptors for the treatment of cancer *Richard Morgan, bluebirdbio, Cambridge, MA* INV060 Systemic virotherapy for multiple myeloma *Stephen Russell, Mayo Clinic, Rochester, MN*
10.00	Morning break
10.30	**Parallel sessions 4a, 4b, 4c, 4d**
Yangtze	**4a: RNA therapeutics** *Chairs: Annemieke Aartsma-Rus, Bruno Pitard* INV061 Beyond clinical trials for antisense-mediated exon skipping for Duchenne Muscular Dystrophy *Annemieke Aartsma Rus, Leiden University Medical Centre* INV062 Pip peptide conjugates of exon skipping PMO for therapy of Duchenne Muscular Dystrophy *Michael Gait, Medical Research Council, Cambridge* *Proffered papers* OR032 Synthetic mRNA encoding vaccinia virus PKR inhibitors improves immunisation with Semliki Forest virus replicons *Tim Beissert, University Medical Center, Mainz* OR033 Messenger RNA in cancer immunotherapy: TriMix delivering an antitumor message *Karine Breckpot, Vrije Universiteit, Brussels* OR034 Best of both worlds – a novel versatile AAV vector toolbox for combinatorial CRISPR and RNAi expression *Dirk Grimm, DZIF, Heidelberg University Hospital* OR035 *In vivo* mRNA introduction using polyplex nanomicelles to treat neurological disorders *Keiji Itaka, University of Tokyo*

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10.30 *Mississippi*	**4b: Gene and cell therapy for metabolic diseases** *Chairs: Gerard Wagemaker, Harald Petri* INV063 Perinatal gene therapy ameliorates a mouse model of neuronopathic Gaucher disease *Simon Waddington, UCL Institute for Women's Health, London* INV064 Toward a curative single intervention therapy in Pompe disease *Niek van Til, University Medical Centre Utrecht* *Proffered papers* OR036 Phase 1 clinical trial of liver directed gene therapy with rAAV5/2-PBGD in acute intermittent porphyria: safety data *Gloria Gonzalez-Aseguinolaza, Centro de Investigación Médica Aplicada (CIMA), Pamplona* OR037 Full correction of neurologic and somatic lysosomal pathology in Mucopolysaccharidosis type IIIB by AAV9-based gene therapy *Virginia Haurigot, Universitat Autònoma de Barcelona* OR038 Development of *ex vivo* gene therapy for familial LCAT deficiency syndrome by self-transplantation of therapeutic-enzyme secreting adipocytes *Masayuki Kuroda, Chiba University* OR039 Lentiviral vector based hematopoietic stem cell gene therapy mediates sustained expression of functional thymidine phosphorylase in a mouse model for mitochondrial neurogastrointestinal encephalomyopathy *Rana Yadak, Erasmus Medical Center, Rotterdam*
10.30 *Auditorium*	**4c: Blood coagulation diseases** *Chairs: Sam Wadsworth, Zoltan Ivics* INV065 Long term safety and efficacy of a novel self-complementary adeno-associated viral vector encoding human FIX in patients with severe hemophilia B *Amit Nathwani, Royal Free NHS Trust; UCL Cancer Institute; NHSBT, London* INV066 Platelet-mediated gene therapy of hemophilia *Shi Qizen, Medical College of Wisconsin, Milwaukee* *Proffered papers* OR040 Safety and efficacy of AAV5-hFIX in non-human primates *Bart Nijmeijer, uniQure BV, Amsterdam* OR041 Baboon envelope pseudotyped LVs mediate high level gene transfer in human B cells allowing secretion of FIX at therapeutic levels in humanised NSG mice *Els Verhoeyen, Université de Lyon-1; CIRI, INSERM U1111; INSERM U1065, Nice*

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	OR042 Targeting FVIII expression to specific cell-types to overcome immunological responses for hemophilia A gene therapy *Antonia Follenzi, Università del Piemonte Orientale, Novara*
10.30 *Amazon*	**4d: Immunotherapy of cancer I** *Chairs: Mirjam Heemskerk, Atilio Bondanza* INV067 PK/PD modeling of CAR-T-cell immunotherapy targeted at the cancer-initiating antigen CD44v6 *Atilio Bondanza, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* INV068 High affinity T-cell receptors for targeting B-cell malignancies *Mirjam Heemskerk, Leiden University Medical Centre* *Proffered papers* OR043 Redirection of CD4+ T-cells with ICOS-based chimeric antigen receptors *Sonia Guedan Carrio, University of Pennsylvania, Philadelphia* OR044 NY-ESO-1-specific Single Edited T-cells efficiently eliminate multiple myeloma without inducing xenogeneic GvHD *Sara Mastaglio, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR045 A traceless selection system that allows efficient generation of auxiliary vector-free Transposon and CRISPR-modified T-cell products *Riccardo Mezzadra, NKI-AvL, Amsterdam* OR046 Donor T-cells for hematopoietic-restricted minor histocompatibility antigens are induced in patients with combined Graft-versus-Leukemia and Graft-versus-Host disease *Margot Pont, Leiden University Medical Centre*
12.30	NVGCT General Assembly – Auditorium
12.30	Lunch – Exhibition and posters
14.00	**5: New approaches to engineering vectors and cells**
Auditorium	*Chairs: Luigi Naldini, Phil Gregory* INV069 Derivatives of human pluripotent stem cells: the new patient? *Christine Mummery, Leiden University Medical Centre* INV070 Genome editing with zinc finger nucleases *Phil Gregory, Sangamo BioSciences, Inc, Richmond, CA* INV071 Novel rAAV vectors for episomal and integration based gene transfer *Mark Kay, Stanford University, CA*

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15.00	**Parallel sessions 5a, 5b, 5c, 5d**
Auditorium	**5a: Primary immune deficiencies** *Chairs: Alessandro Aiuti, Harry Malech* INV072 Progress in gene therapy for PIDs *Adrian Thrasher, UCL Institute of Child Health, London* INV073 Update on gene therapy trials for severe combined immunodeficiency and Wiskott-Aldrich Syndrome *David Williams, Boston Children's Hospital, Harvard Medical School, Boston, MA* *Proffered papers* OR047 Genetic correction of induced pluripotent stem cells from a Wiskott-Aldrich Syndrome patient normalizes the immune defects *Brian Davis, University of Texas, Houston* OR048 *Ex vivo* Gene Therapy in a Mouse Model of Leukocyte Adhesion Deficiency Type I *Elena Almarza, CIEMAT/CIBERER, Madrid* OR049 Finding levels of RAG1 expression that will correct RAG1 Severe Combined Immunodeficiency (Final) *Karin Pike-Overzet, Leiden University Medical Centre* OR050 Transplantation of human SCID stem cells in NSG mice gives new insights into human T-cell development and reveals where SCID mutations act *Anna-Sophia Wiekmeijer, Leiden University Medical Center*
15.00 *Mississippi*	**5b: Gene and cell therapy for neural diseases** *Chairs: Joost Verhaagen, Nathalie Cartier* INV074 Gene therapy strategies for Alzheimers disease *Nathalie Cartier, Université Paris Sud; INSERM U986; CEA MIRcen* INV075 Efficient lentiviral vector-mediated delivery of hARSA in the brain of juvenile non-human primates *Angela Gritti, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* *Proffered papers* OR051 New model for Ubiquilin2-linked ALS: investigation of pathological mechanisms and therapeutic perspectives *Maria-Grazia Biferi, Institut de Myologie, INSERM-CNRS-UPMC-UMR 974, Paris*

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	OR052 High level expression of human iduronidase throughout the brain in a murine model of mucopolysaccharidosis type I (MPS I) after non-invasive AAV-mediated gene delivery to the CNS *Karen Kozarsky, ReGenX Biosciences, Washington DC* OR053 OXB-102: an enhanced gene therapy for Parkinson's disease *Kyriacos Mitrophanous, Oxford BioMedica Ltd* OR054 AAV-mediated delivery of SMN1 in a mouse model of spinal muscular atrophy *Martine Barkats, Institut de Myologie; UPMC-AIM UMR S974, INSERM U 974, CNRS FRE 3617, Paris 6*
15.00 *Yangtze*	**5c: RNA vectors** *Chairs: Sarah Ferber, Manfred Schmitt* INV076 HIV-1 capsid controls tropism through evasion of innate sensors *Greg Towers, UCL, London* INV077 Defining the integration landscape of viral vectors in the era of next generation sequencing *Manfred Schmidt, National Center for Tumor Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg* *Proffered papers* OR055 Rescue of splicing-mediated intron loss maximizes expression in lentiviral vectors containing the human ubiquitin C promoter *Aaron Cooper, University of California Los Angeles (UCLA)* OR056 Development of F/HN pseudotyped Lentivirus for airway gene transfer *Deborah Gill, UK Cystic Fibrosis Gene Therapy Consortium, Oxford, Edinburgh & London* OR057 BET-independent MLV-based vectors target away from promoters and regulatory elements *Sara El Ashkar, KU Leuven* OR058 Measles virus glycoprotein pseudotyped lentiviral vectors transduce HSC SCID repopulation cells at efficiencies reaching up to 100% *Camille Lévy, Université de Lyon-1; CIRI, INSERM U1111*
15.00 *Amazon*	**5d: Bioprocessing of cell and gene therapy products** *Chairs: Otto Merten, James Miskin* INV078 A new downstream process for large scale manufacturing of AAV9 vectors *Matthias Hebben, Généthon, Evry* INV079 High titer production of GMP grade SIN gamma-retroviral vectors *Klaus Kühlcke, Eufets, Idar-Oberstein*

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	Proffered papers OR059 Development of the manufacturing process for the *ex vivo* gene therapy for ADA-SCID (GSK2696273): process design, validation and comparability *Nina Kotsopoulou, GlaxoSmithKline, Stevenage* OR060 GLP preclinical studies for gene therapy medicinal products combine highest regulatory standards with outstanding scientific significance *Aisha Sauer, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR061 Automated magnetic isolation and lentiviral vector modification of human CD34+ cells in a functionally closed system *Ian Johnston, Miltenyi Biotec GmbH, Bergisch Gladbach* OR062 High yield adenovirus production in fixed-bed bioreactor *Hanna Lesch, FKD Therapies; FinVector Vision Therapies, Kuopio*
17.00	Afternoon break
17.30	**Parallel sessions 6a, 6b, 6c, 6d**
Amazon	**6a: Regenerative medicine** *Chairs: Joost Verhaagen, Nathalie Cartier* INV080 Generation of dopamine neurons for cell therapy in Parkinson's disease *Malin Parmar, Lund University* INV081 Gene therapy for neurotrophic factors to promote regeneration of injured peripheral nerves *Joost Verhaagen, Netherlands Institute for Neuroscience, Amsterdan* INV082 Stem cells based technologies as non-viral transfection approaches *Marcelle Machluf, Technion- Israel Institute of Technology, Haifa* *Proffered papers* OR063 Phase 1-2 clinical trial in patients with decompensated liver cirrhosis treated with bone-marrow derived endotelial progenitor cells: preliminary safety and efficacy analysis *Delia D'Avola, Clinica Universidad de Navarra, Pamplona* OR064 Advanced tuning of Notch signaling to regulate *in vivo* myogenic repair of murine and human mesoangioblasts *Mattia Quattrocelli, KU Leuven*

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17.30 *Yangtze*	**6b: Inflammatory and autoimmune diseases** *Chairs: Margriet Vervoordeldonk, Rosa Bacchetta* INV083 Adult stem cells organoids and regenerative medicine *Robert Vries, University Medical Centre, Utrecht* INV084 Generation of regulatory T-cells by FOXP3 gene transfer: different approaches towards CD4FOXP3 T-cell-based therapy for autoimmune diseases *Rosa Bacchetta, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* *Proffered papers* OR065 Antigen-specific myeloid-derived suppressor cells ameliorate experimental autoimmune encephalomyelitis *Silvia Casacuberta-Serra, Universitat Autònoma de Barcelona* OR066 Modulation of the ATP:adenosine balance by AAV-5 mediated gene delivery of CD39-CD73 in fibroblast-like synoviocytes from rheumatoid arthritis patients *Susanne Snoek, Arthrogen BV, Amsterdam* OR067 Modulation of mouse models of rheumatoid arthritis by systemic delivery of immunoregulatory bovine milk derived exosomes *Fons van de Loo, Radboud University Medical Center, Nijmegen*
17.30 *Auditorium*	**6c: Oncolytic therapies** *Chairs: Len Seymour, Victor van Beusechem* INV085 Armed oncolytic adenovirus can overcome critical obstacles in adoptive T-cell therapy of solid tumors *Akseli Hemminki, University of Helsinki, Finland* INV086 Preliminary results of the phase 1 trial with the Delta24RGD oncolytic adenovirus, administered by CED in patients with recurrent Glioblastoma *Clemens Dirven, Erasmus Medical Centre, Rotterdam* *Proffered papers* OR068 Immune checkpoint blockade enhances oncolytic Measles virus therapy *Christine Engeland, National Center for Tumour Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg*

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	OR069 Decapitated reoviruses with a little iLOV *Diana van den Wollenberg, Leiden University Medical Centre* OR070 Insertion of an albumin-binding domain in adenovirus hexon improves the pharmacokinetics and antitumor efficacy of oncolytic adenoviruses *Luis Alfonso Rojas, Instituto Catalan de Oncología-IDIBELL, L´Hospitalet de Llobregat* OR071 Immunotherapy against pancreatic cancer using sequential administration of antigenically distinct oncolytic viruses expressing oncostatin M *Ruben Hernandez-Alcoceba, Centro de Investigación Médica Aplicada (CIMA), Pamplona*
17.30 *Mississippi*	**6d: Immunotherapy of Cancer II** *Chairs: Zoltan Ivics, Laurence Cooper* INV087 Human translation of Sleeping Beauty system and next-generation clinical trials *Laurence Cooper, MD Anderson Cancer Centre, Houston, TX* *Proffered papers* OR072 An innovative CAR-T-cell spacer allowing selection/tracking and enabling superior antitumor effects *in vivo* *Monica Casucci, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR073 TAP-independent presentation of exogenous epitopes by oncolytic adenoviruses enhances specific immune responses and antitumor activity *Alba Rodríguez-García, Instituto Catalan de Oncología-IDIBELL, L´Hospitalet de Llobregat* OR074 Alpharetroviral vectors for the transduction of primary human natural killer cells: selective enhancement of tumor cytotoxic activity by chimeric antigen receptors *Julia Suerth, Hannover Medical School* OR075 Targeted *in vivo* TCR gene transfer into mouse T-cell subsets for immunotherapy of cancer *Wolfgang Uckert, Humboldt University, Berlin* OR076 Re-engineering of human CYP4B1 for optimal catalytic processing of 4-ipomeanol and use as a suicide gene in adoptive cell therapy *Constanze Wiek, Heinrich Heine University, Düsseldorf* OR077 Long-term episomal gene transfer for safe engineering of T-cells for adoptive cell therapy of cancer *Di Yu, Uppsala University*

PLEASE NOTE: At 2am last night the clocks turned back one hour for the end of Daylight Saving Time

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MAIN CONGRESS	
09.00	**Parallel sessions 7a, 7b, 7c, 7d**
Yangtze	**7a: Exosomes and non-viral delivery** *Chairs: Fons van de Loo, Enrico Mastrobattista* INV088 Clinical potential of cell-derived vesicles: challenges to analyse the molecular composition of the extracellular vesicle pool *Marca Wauben, Utrecht University* INV089 Extracellular vesicles for therapeutic RNA delivery: Promises and pitfalls *Pieter Vader, University Medical Centre, Utrecht* *Proffered papers* OR078 Gene therapy based on cytidine deaminase-targeting overcomes pancreatic cancer resistance to chemotherapy *Marion Gayral, Paul Sabatier University; INSERM U1037, Toulouse* OR079 Results of a phase IIb non-viral gene therapy trial from the UK CF Gene Therapy Consortium *Uta Griesenbach, UK Cystic Fibrosis Gene Therapy Consortium, Oxford, Edinburgh & London* OR080 Virus-free delivery of microRNA into freshly isolated patient derived CD105+ MSCs using a novel magnet-bead based vector system *Paula Müller, University of Rostock* OR094 Bio-inspired approaches for siRNA delivery *Koen Raemdonck, Ghent University*
09.00 *Amazon*	**7b: Infectious diseases** *Chairs: Ben Berkhout, Luc van der Laan* INV090 Preclinical studies on Newcastle disease virus for the treatment of pancreatic adenocarcinoma *Ron Fouchier, Erasmus Medical Centre, Rotterdam* INV091 RNAi -based gene therapy for HIV-1 *Ben Berkhout, University of Amsterdam* *Proffered papers* OR081 A therapeutic anti-Hepatitis C virus shmiRNA integrated into the miR-122 genomic locus mediates a potent anti-viral response *Elena Senis, DZIF, Heidelberg University Hospital*

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	OR082 Integration driven HIV-1/STAT5B chimeric transcripts confer a selective advantage to blood cells in patients under anti-retroviral therapy *Daniela Cesana, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR083 Cross-Clade inhibition of HIV on primary cells by CXCR4 or CCR5 fused to the C34 peptide from gp41 HR2 *Michael Holmes, Sangamo BioSciences, Inc, Richmond, CA*
09.00 *Auditorium*	**7c: Gene Editing II** *Chairs: Toni Cathomen, Christof von Kalle* INV092 Gene editing in pluripotent stem cells to model primary immune deficiencies *Toni Cathomen, University Medical Centre, Freiburg* INV093 Targeted editing of the human (epi)genome using artificial transcriptional repressors *Angelo Lombardo, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* *Proffered papers* OR001 A CRISPR way to eradicate latent HIV: Targeted excision of integrated HIV-1 DNA from human cells through vector-based genome engineering *Kathleen Börner, DZIF, Heidelberg University Hospital* OR002 An AAV vector toolbox for CRISPR/Cas9-mediated genome engineering *Polychronis Fatouros, DZIF, Heidelberg University Hospital* OR003 Adenoviral vector DNA is a preferred homologous recombination substrate for accurate genome editing using engineered nucleases *Ignazio Maggio, Leiden University Medical Center* OR004 Engineering human models of tumor-associated chromosomal translocations with the RNA-guided CRISPR–Cas9 system *Raúl Torres, Centro Nacional de Investigaciones Cardiovasculares (CNIC), Madrid*

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09.00 *Mississippi*	**7d: Hematopoietic stem cell and HSC gene therapy** *Chairs: Juan Bueren, Nathalie Cartier* INV094 Outcomes of gene therapy for b-Thalassemia Major via transplantation of autologous hematopoietic stem cells transduced *ex vivo* with a lentiviral bA-T87Q-Globin vector *Salima Hacein-Bey-Abina, Hôpital Universitaire Necker – Enfants Malades, Paris* *Proffered papers* OR088 A non human primate model for autologous transplantation of IPSC-derived hematopoietic cells *Leila Maouche-Chrétien, University Paris Sud 11; CEA-iMETI, INSERM U962, Fontenay aux Roses* OR089 Exploring bone marrow microenvironment in a murine model of ß-thalassemia *Annamaria Aprile, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR090 Mesenchymal stromal cells enhance the engraftment of low numbers of hematopoietic stem cells in a mouse model of autologous transplantation *Maria Fernandez-Garcia, CIEMAT/CIBERER, Madrid* OR091 Retroviral expression of dominant-negative Mpl disrupts THPO/MPL signaling and HSC maintenance *Saskia Kohlscheen, Paul-Ehrlich-Institute, Langen* OR092 CD34+ cells isolated from different sources: exploring their biology for future clinical perspectives *Maria Rosa Lidonnici, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* OR093 Defining regulatory elements in human embryonic and somatic stem cells by MLV integration profiling *Valentina Poletti, Généthon, Evry*
11.00	Morning break

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SUNDAY 26 OCTOBER 2014

11.30	**Presidential symposium and awards ceremony**
Auditorium	*Chairs: Luigi Naldini, Gerard Wagemaker* INV097 Keynote lecture; Nobel Laureate for Medicine *Shinya Yamanaka, Centre for iPS Cell Research and Application, Kyoto University* INV098 The innovative medicines: a European engine for stem cell research *Michel Goldman, Innovative Medicines Initiative (IMI), Brussels* INV099 StemBANCC – Patient iPSCs to study a wide range of diseases *Martin Graf, Hoffmann-La Roche Ltd, Basel* INV100 EBiSC: the European bank for induced pluripotent stem cells *Timothy Allsopp, Pfizer Ltd, Ipswich* **Young Investigator Awards** OR101 MiRNAs 182 and 183 are necessary to maintain adult cone photoreceptor outer segments and visual function *Volker Busskamp, Centre for Regenerative Therapies, Dresden* OR102 Development of oncolytic vaccines for cancer treatment: the past, the present and the future! *Vincenzo Cerullo, University of Helsinki* **Oustanding Achievement Award** INV103 Gene therapy of chronic granulomatous disease: lessons learned and future perspectives *Manuel Grez, Institute for Biomedical Research, Georg-Speyer-Haus, Frankfurt* **Presentation of new president and 2015 Congress**