INV Invited speaker • OR Selected abstracts • P Lightening talks

Programme Tuesday 17 October 2017

17:00 - 19:00

1: ESGCT 2017 opening

Room C01

Chairs Robin Ali, Zoltan Ivics

ESGCT 25th Anniversary retrospective
Robin Ali [UNIVERSITY COLLEGE LONDON]

OxfordBioMedica

INV29 **Genotoxicity – 15 years after, and the future?**
Christopher Baum [HANNOVER MEDICAL SCHOOL]

INV30 **Clinical gene therapy for neurodegenerative diseases: Past, present, and future**
Nathalie Cartier [INSERM/ CEA UMR1169, MIRCEN CEA AND UNIVERSITY PARIS-SUD, UNIVERSITY PARIS SACLAY]

INV31 **Haemophilia: From Talmud to CRISPR/Cas**
Thierry vandenDriessche [FREE UNIVERSITY OF BRUSSELS]

19:00 - 21:00 : Welcome reception | **Molecular Therapy meet the editor**

Programme Wednesday 18 October 2017

08:30 - 10:40

1a: Disease modelling

Room B05 - B07

Chairs Robin Ali, Andras Nagy

INV32 **Astrocyte - neuronal cross talk in neurodegeneration**
Siddharthan Chandran [UNIVERSITY OF EDINBURGH]

INV33 **Human glial progenitor cell-based treatment and modelling of neurological disease**
Steve Goldman [UNIVERSITY OF ROCHESTER MEDICAL CENTRE, NY]

OR01 **Generation of three-dimensional human artificial skeletal muscle tissue from iPS cells enables complex disease modelling for muscular dystrophy**
Francesco Saverio Tedesco [UNIVERSITY COLLEGE LONDON]

OR02 **Reprogramming triggers mobilisation of endogenous retrotransposons in human induced pluripotent stem cells with genotoxic effects on host gene expression**
Gerald Schumann [PAUL EHRLICH INSTITUTE, LANGEN]

OR03 **Dynamic remodelling of neural cellular and extracellular signatures depicted in 3D *in vitro* differentiation of human iPSC-derived NSC**
Daniel Simão [IBET, UNIVERSIDADE NOVA DE LISBOA, OEIRAS]

OR04 **Development of novel AAV variants with superior photoreceptor transduction properties**
Stylianos Michalakis [LUDWIG MAXIMILIAN UNIVERSITY, MUNICH]

Programme Wednesday 18 October 2017

08:30 - 10:40

1a: Disease modelling continued

Room B05 - B07

P117 **Characterisation of iPSCs from a patient with transposition of the great arteries**
Akaitz Dorronsoro González [MEDICAL RESEARCH INSTITUTE, LA FE, VALENCIA]

P119 **Characterisation of type I interferon responses to viral vectors in human macrophages**
Maria Primo [WELLCOME TRUST SANGER INSTITUTE, CAMBRIDGE]

1b: Ocular and central nervous system gene and cell therapy I

Room C01

Chairs Nicole Déglon, Nathalie Cartier

Voyager THERAPEUTICS

INV34 **Gene therapy approach for the treatment of Alzheimer's disease: Targeting apolipoprotein E with novel AAV vectors delivered systemically**
Eloise Hudry [MASSACHUSSETTS GENERAL HOSPITAL, HARVARD, MA]

INV35 **Too little vs. too late in retinal gene therapy**
Stephen Tsang [COLUMBIA UNIVERSITY, NY]

OR05 **AAV-mediated CYP46A1 gene therapy for Huntington's disease**
Nathalie Cartier [INSERM/ CEA UMR1169, MIRCEN CEA AND UNIVERSITY PARIS-SUD, UNIVERSITY PARIS SACLAY]

OR06 **Gradual improvements in the motor and cognitive function after gene therapy for patients with AADC deficiency**
Karin Kojima [JICHI MEDICAL UNIVERSITY]

OR07 **Efficacy and safety of ocular AAV mediated optogenetic therapy for retinitis pigmentosa in rd1 mice and non-human primates support the first-in-human clinical trial of GS030**
Anne Douar [GENSIGHT BIOLOGICS, PARIS]

OR08 **Long-term expression of secreted-klotho protects against cognitive decline in aged animals**
Miguel Chillon Rodriguez [VALL D'HEBRON RESEARCH INSTITUTE (VHIR), BARCELONA]

P223 **AAV5-miHTT gene therapy demonstrates sustained huntingtin lowering and functional improvement in Huntington disease mouse models**
Lisa Spronck [UNIQURE BIOPHARMA B.V., AMSTERDAM]

P211 **Anti-transgene cellular immune reponses can be induced by subretinal gene transfer with rAAV in a dose-dependent manner**
Sylvain Fisson [INSERM/GENETHON, EVRY]

INV Invited speaker • OR Selected abstracts • P Lightening talks

Programme Wednesday 18 October 2017

08:30 - 10:40

1c: Cancer gene therapy I

Room B08 - B09

Chairs Pierre Cordelier, Vincenzo Cerullo

INV36 **Targeting T-cells to human cancer cells using an oncolytic virus expressing bispecific T-cell engagers**
Len Seymour [UNIVERSITY OF OXFORD]

INV37 **Clinical translation of retroviral replicating vector-mediated gene therapy for cancer**
Noriyuki Kasahara [UNIVERSITY OF MIAMI, FL]

OR09 **Oncolytic measles viruses as therapeutic vectors for targeted BiTE expression in solid tumours**
Tobias Speck [DKFZ / NCT, HEIDELBERG]

OR10 **An oncolytic recombinant measles virus is a candidate of a novel therapeutic agent for triple negative breast cancer**
Tomoko Fujiyuki [THE UNIVERSITY OF TOKYO]

OR11 **Efficacy, safety, and covariates of outcomes with axicabtagene ciloleucel (axi-cel; KTE-C19) from ZUMA-1, a pivotal trial in patients with refractory, aggressive non-Hodgkin lymphoma (NHL)**
Yi Lin [MAYO CLINIC, ROCHESTER, MN]

P021 **Bioselection of an adenovirus encoded microRNA library identifies miR-99b and miR-485 as enhancers of adenoviral oncolysis in pancreatic cancer**
Maria Rovira Rigau [IDIBAPS, BARCELONA]

P043 **T cells expressing CAR harbouring GITR signalling domain exhibit prolonged *in vivo* survival and resistance to immunosuppression that lead to efficient tumour eradication**
Takuma Kato [MIE UNIVERSITY]

P023 **PeptiENV: A novel cancer immunotherapy platform based on enveloped viruses coated with tumour-specific MHC-I restricted peptides**
E Ylösmäki [UNIVERSITY OF HELSINKI]

P045 **Gamma-delta CAR-T cells as an off-the-shelf cellular immunotherapy platform for paediatric B-cell lymphoblastic leukaemia**
Matteo Doglio [SAN RAFFAELE HOSPITAL AND VITA-SALUTE SAN RAFFAELE UNIVERSITY, MILAN]

10:40 - 11.10 COFFEE BREAK

Programme Wednesday 18 October 2017

11:10 - 13.10

2: Highlights of clinical progress I

Room C01

Chairs Alessandro Aiuti; Juan Bueren

INV38 **New directions in CAR therapy**
Michel Sadelain [MEMORIAL SLOAN KETTERING CANCER CENTRE, NEW YORK, NY]

INV39 **Replicating viruses as biological machines to treat cancer**
John Bell [UNIVERSITY OF OTTAWA AND BIOCANRX, ON]

INV40 **Gene therapy in severe immunodeficiency**
Adrian Thrasher [UNIVERSITY COLLEGE LONDON]

INV41 **AVXS-101 phase 1 gene therapy clinical trial in SMA type 1: Event free survival and achievement of developmental milestones**
Jerry Mendell [NATIONWIDE CHILDREN'S HOSPITAL, COLUMBUS, OH]

13:10 - 14:40 LUNCH

13:30 - 14:30 REGULATORY WORKSHOP (LUNCH PROVIDED)
Room B05 - B07

Matthias Renner [PAUL EHRLICH INSTITUTE, LANGEN] **Introduction**
Martin Wisher [MERCK] **Regulatory trends for the testing of ATMPs - EU and USA perspective on quality control testing**

14:40 - 16:50

2a: Ocular and central nervous system gene and cell therapy II

Room B05 - B07

Chairs Alberto Auricchio, Eloise Hudry

INV42 **The self-inactivating KamiCas9 system for the editing of CNS disease genes**
Nicole Déglon [LAUSANNE UNIVERSITY HOSPITAL (CHUV)]

INV43 **Gene therapy treatment of congenital deafness in a mouse model of Usher syndrome type IC**
Gwenaëlle Geleoc [HARVARD CHILDREN'S HOSPITAL, CAMBRIDGE, MA]

OR12 **Intraputaminal AADC gene therapy for advanced Parkinson's disease: Interim results of a phase 1b Trial**
Bernard Ravina [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

OR13 **Dynorphin-based "drug on demand" gene therapy suppresses seizures and restores lost brain functions in drug-resistant temporal lobe epilepsy**
Regine Heilbronn [CHARITÉ MEDICAL UNIVERSITY BERLIN]

INV Invited speaker • OR Selected abstracts • P Lightening talks

Programme Wednesday 18 October 2017

14:40 - 16:50

2a: Ocular and central nervous system gene and cell therapy II continued

Room B05 - B07

OR14 Correction of sensory ataxia in a novel mouse model of Friedreich's ataxia using gene therapy approach
Françoise Piguet [IGBMC, STRASBOURG]

OR15 Gene therapy with AAV-CDKL5 vectors in models of CDKL5 disorder
Yunan Gao [IMPERIAL COLLEGE, LONDON]

P213 Translation of intrathecal delivery of an AAV gene therapy targeting SOD1 for the treatment of ALS
Dinah Sah [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

P221 AAV-CYP46A1 is beneficial in Alzheimer's disease: From mice to non-human primates
Sandro Alves [BRAINVECTIS THERAPEUTICS, FONTENAY-AUX-ROSES]

2b: Gene editing I

Room C01

Chairs Toni Cathomen, Adrian Thrasher

MaxCyte®

INV44 Genome editing: From modelling disease to novel therapeutics
Chad Cowan [HARVARD STEM CELL INSTITUTE, CAMBRIDGE, MA]

INV45 Gene and mutation independent therapy via CRISPR/Cas9 mediated cellular reprogramming in rod photoreceptors
Kang Zhang [UC SAN DIEGO, CA]

OR16 *In vivo* genome editing via non-viral delivery of zinc finger nucleases enables supraphysiological levels of therapeutic proteins and greater than 90% protein knockdown of multiple therapeutic gene targets via targeted integration and NHEJ, respectively, in wild type mice
Anthony Conway [SANGAMO THERAPEUTICS, RICHMOND, CA]

OR17 CRISPR/Cas9-mediated editing for dominant genetic disorders: Efficient excision of trinucleotide repeat expansion in myotonic dystrophy
Sumitava Dastidar [FREE UNIVERSITY OF BRUSSELS]

Programme Wednesday 18 October 2017

14:40 - 16:50

2b: Gene editing I continued

Room C01

OR18 Direct correction of Fanconi anaemia associated mutations in haematopoietic stem and progenitor cells by a novel NHEJ-mediated gene editing approach
Francisco José Román Rodríguez [CIEMAT/CIBERER-ISCIII, MADRID]

OR19 Targeted genome editing of recombination activating gene 1 to potentially treat severe combined immunodeficiency
Nicolò Sacchetti [HSR TIGET, MILAN]

P129 GeneRide™: Therapeutic *in vivo* gene targeting without nucleases
Adi Barzel [LOGICBIO THERAPEUTICS INC., CAMBRIDGE, MA]

P131 Targeted gene correction of Wiskott-Aldrich syndrome in human haematopoietic stem and progenitor cells
Alessia Cavazza [UNIVERSITY COLLEGE LONDON]

2c: Muscle and pulmonary gene and cell therapy

Room B08 - B09

Chairs Olivier Danos, Uta Griesenbach

GENETHON CURE THROUGH INNOVATION

INV46 Development of muscle-directed gene therapy using tissue specific transcriptional modules identified by genome-wide computational analysis to maximise gene expression in skeletal muscle, heart and diaphragm
Marinee Chuah [FREE UNIVERSITY OF BRUSSELS]

INV47 Gene therapy for cystic fibrosis: An update
Eric Alton [IMPERIAL COLLEGE LONDON]

INV48 AAV-mediated gene therapy for muscular dystrophy
George Dickson [ROYAL HOLLOWAY, LONDON]

OR20 "Silence and Replace": Development of a single AAV vector system for the treatment of oculopharyngeal muscular dystrophy (OPMD)
Vanessa Strings-Ufombah [BENITEC BIOPHARMA, SYDNEY, NSW]

OR21 Combined Notch and PDGF signalling in muscle satellite cells induces a pericyte-like phenotype with potential cell therapy relevance
Louise Moyle [UNIVERSITY COLLEGE LONDON]

16:50 - 17:20 COFFEE BREAK

Main Congress

INV Invited Speaker • OR Proffered Papers • P Lightening Talks

Programme Wednesday 18 October 2017

17:20 - 18:20

3: Stem cells: Biology, manipulation and reprogramming

Room C01

Chairs Christof von Kalle, Luigi Naldini

bluebirdbio

INV49 **Defining and providing a solution for cell therapy safety**
Andras Nagy [LUNENFELD-TANENBAUM RESEARCH INSTITUTE, TORONTO, ON]

INV50 **Highly efficient genome editing by homologous recombination of haematopoietic stem cells with the goal to cure genetic diseases of the blood and immune system**
Matthew Porteus [STANFORD UNIVERSITY, CA]

INV51 **Reprogramming, gene editing stem cells and organ generation: *In vitro* and *in vivo* approaches to increase healthspan**
Juan Carlos Izpisua-Belmonte [SALK INSTITUTE, LA JOLLA, CA]

INV52 **Demolishing cell reprogramming barriers in ageing and progeria**
Carlos Lopez-Otin [UNIVERSITY OF OVIEDO]

18.30 - 21.00 : Poster session 1 (Please see page 87)

Notes:

Notes:

INV Invited speaker • OR Selected abstracts • P Lightening talks

Programme Thursday 19 October 2017

08:00 - 10:10

3a: Vector development I

Chairs Hildegard Büning, Boris Fehse

Biogen

INV53 From a cancer causing virus to a potentially safer strategy for gene therapeutics
Axel Schambach [HANNOVER MEDICAL SCHOOL]

INV54 Transformer adenovirus for enhanced bioproduction of AAV and recombinant proteins
Weiheng Su [UNIVERSITY OF OXFORD]

OR22 Divergent AAV serotypes demonstrate an alternate, AAVR independent, entry pathway
Amanda Dudek [HARVARD UNIVERSITY, BOSTON, MA]

OR23 Ad3.0: An engineered adenovirus library to significantly expand the spectrum of available vector types and their applications
Anja Ehrhardt [WITTEN/HERDECKE UNIVERSITY]

OR24 Generation of highly variably AAV capsid libraries using a novel DNA shuffling platform
Marti Cabanes Creus [CHILDREN'S MEDICAL RESEARCH INSTITUTE, SYDNEY, NSW]

OR25 Enhancement of titres for vectors encoding miRNA adapted shRNAs (shRNAmiRs) associated with increased viral genomic transcripts via Drosha microprocessor knockout (KO) in producer cells
Hee Ho Park [BOSTON CHILDREN'S HOSPITAL, MA]

P334 Large-scale production of lentiviral vectors for use in therapeutic applications
Yatish Lad [OXFORD BIOMEDICA]

P318 A screening procedure identifying DARPins suitable for the retargeting of lentiviral and AAV vectors
Jessica Hartmann [PAUL EHRLICH INSTITUTE, LANGEN]

Room B05 - B07

Programme Thursday 19 October 2017

08:00 - 10:10

3b: Regenerative therapies

Chairs Siddharthan Chandran, Robin Ali

INV55 Inflammation and regeneration
Hans Dieter Volk [CHARITÉ MEDICAL UNIVERSITY BERLIN]

INV56 Ageing and myelin regeneration in the CNS
Robin Franklin [UNIVERSITY OF CAMBRIDGE]

INV57 Cell therapy for muscular dystropy: Road towards efficacy
Giulio Cossu [UNIVERSITY OF MANCHESTER]

OR26 Phase I/IIa clinical trial for recessive dystrophic epidermolysis bullosa using EB-101 (COL7A1 gene-corrected autologous keratinocytes)
JY Tang [STANFORD UNIVERSITY, CA]

OR27 Gene editing-mediated excision of mutation-bearing exon 80 of COL7A1 gene for the efficient correction of recessive dystrophic epidermolysis bullosa patient derived-epidermal stem cells
Jose Bonafont Arago [CARLOS III UNIVERSITY (UC3M), MADRID]

Room C01

Notes:

Programme Thursday 19 October 2017

08:00 - 10:10

3c: Metabolic and lysosomal storage diseases

Room B08 - B09

Chairs Fatima Bosch, Alberto Auricchio

INV58 **Haematopoietic stem cell based gene therapy for the treatment of lysosomal storage disorders**
Alessandra Biffi [DANA-FARBER/BOSTON CHILDREN'S CANCER AND BLOOD DISORDERS CENTRE]

INV59 **Gene therapy for liver inherited metabolic diseases**
Gloria Aseguinola [FIMA PAMPLONA]

OR28 **A phase 1/2 clinical trial of systemic gene transfer of scAAV9.U1a.hSGSH for MPS IIIA: Safety, tolerability, and preliminary evidence of biopotency**
Kevin Flanigan [NATIONWIDE CHILDREN'S HOSPITAL, COLUMBUS, OH]

OR29 **Correction of hereditary tyrosinemia type 1 in a large animal model through *in vivo* liver-directed lentiviral gene therapy**
Clara Nicolas [MAYO CLINIC, ROCHESTER, MN]

OR30 **CRISPR/Cas mediated disruption of the glycolate oxidase gene is an efficacious treatment for primary hyperoxaluria type I**
Nerea Zabaleta Lasarte [CIMA, UNIVERSITY OF NAVARRA, PAMPLONA]

OR31 **Whole-body correction of Pompe disease by AAV liver-mediated gene transfer of engineered secretable GAA transgenes**
Francesco Puzzo [GENETHON AND INSERM U951, EVRY]

P190 **Correction of storage disease in MPS I mice by adoptive transfer of human B cells engineered for human iduronidase expression using the Sleeping Beauty transposon system**
R. Scott McIvor [IMMUSOFT CORPORATION, SEATTLE, WA]

P186 **Correction of visual and auditory function by AAV9-mediated gene therapy in a mouse model of mucopolysaccharidosis type IIIB**
Albert Ribera [AUTONOMOUS UNIVERSITY OF BARCELONA-CBATEG]

10:10 - 10:40 COFFEE BREAK

Programme Thursday 19 October 2017

10:40 - 12:40

4: Highlights of clinical progress II

Room C01

Chairs Amit Nathwani, Bernd Gänsbacher

INV60 **Gene therapy for beta-thalassemia: Update from TIGET BTHAL clinical trial**
Giuliana Ferrari [HSR TIGET, MILAN]

INV61 **Engraftment and repopulation advantage of gene-corrected haematopoietic stem cells in Fanconi anaemia patients**
Juan Bueren [CIEMAT/CIBERER-ISCIII, MADRID]

INV62 **Combined *ex vivo* cell and gene therapy of junctional epidermolysis bullosa by transgenic epidermal stem cells**
Michele De Luca [CENTRE FOR REGENERATIVE MEDICINE "STEFANO FERRARI"]

INV63 **Successful use of haematopoietic stem cell gene therapy to arrest progression of cerebral adrenoleukodystrophy: Results of an international phase 2/3 trial**
David Williams [HARVARD CHILDREN'S HOSPITAL, CAMBRIDGE, MA]

12:40 - 14:10 LUNCH

European Research Council
Established by the European Commission

13:00-14:00 ERC CAREER'S WORKSHOP (LUNCH PROVIDED)
Janka Mátrai [ERC EXECUTIVE AGENCY, BRUSSELS]
The ERC and advanced therapies: How we contribute to each other's success
Case studies: Federico Mingozzi [GENETHON, EVRY], Alberto Auricchio, [TIGEM, NAPLES], Alessandra Biffi [DANA-FARBER, BOSTON]

Room B05 - B07

14:10 - 16:10

4a: Genotoxicity

Room B05 - B07

Chairs Christof von Kalle, Luca Biasco

INV64 **The hidden effects of insertional mutagenesis, beyond oncogenesis**
Eugenio Montini [HSR TIGET, MILAN]

OR32 **Dynamics of haematopoietic reconstitution in a mouse model of haematopoietic stem cell-gene therapy by longitudinal integration site analyses**
Daniela Cesana [HSR TIGET, MILAN]

OR33 **Abnoba-Seq: A highly sensitive and unbiased *in vitro* assay to profile CRISPR/Cas nuclease off-target activity**
Simone Haas [UNIVERSITY OF FREIBURG]

Programme Thursday 19 October 2017

14:10 - 16:10

4a: Genotoxicity continued

OR34 Assessment of the integration profile of self-complementary AAV vector over time in non-human primates
Jenny McIntosh [UNIVERSITY COLLEGE LONDON]

OR35 Recombinant AAV persistence in peripheral blood and bone marrow: Insights from clinical and preclinical studies
Irene Gil-Farina [DKFZ / NCT, HEIDELBERG]

Room B05 - B07

4b: Blood disorders

Chairs David Williams, Adrian Thrasher

INV65 Gene therapy for Wiskott-Aldrich syndrome
Alessandro Aiuti [HSR TIGET, MILAN]

INV66 Haematopoietic stem cell modification for platelet directed gene therapy
Ute Modlich [PAUL EHRLICH INSTITUTE, LANGEN]

INV67 Update on SPK-9001 – an adeno-associated virus mediated gene transfer for haemophilia B
Federico Mingozzi [GENETHON, EVRY]

OR36 Liver-directed gene therapy for haemophilia B with immune stealth lentiviral vectors
Michela Milani [HSR TIGET, MILAN]

OR37 Multi-parametric whole blood dissection: A one shot comprehensive picture of the human haematopoietic system
Luca Basso-Ricci [HSR TIGET, MILAN]

P008 BaEV-LVs transduce HSCs-derived human progenitor T cells, allowing accelerated T cell reconstitution *in vivo* and correction of X-SCID progenitor T cells
Els Verhoeyen [CIRI; INSERM U1111AND C3M INSERM U1065, NICE]

P010 Towards the gene therapy of LAD-I immunodeficient patients
Elena Almarza Novoa [CIEMAT/CIBERER-ISCIII, MADRID]

Room C01

Programme Thursday 19 October 2017

14:10 - 16:10

4c: Vector development II

Chairs Axel Schambach, George Dickson

INV68 Tailoring the AAV vector system for liver-directed gene therapy
Hildegard Büning [HANNOVER MEDICAL SCHOOL]

INV69 Changing lentiviral vector tropism through pseudotyping with viral glycoproteins results in powerful tools for therapeutic gene delivery in haematopoietic cells
Els Verhoeyen [CIRI; INSERM U1111 AND C3M INSERM U1065, NICE]

INV86 Engineering measles virus for cancer therapy
Guy Ungerechts [NCT, HEIDELBURG]

OR38 Transient retrovirus-based CRISPR/Cas9 all-in-one particles for efficient, targeted gene knockout
Melanie Galla [HANNOVER MEDICAL SCHOOL]

OR39 New chimeric gene therapy vectors based on five different mammalian bocaviruses
Julia Fakhiri [HEIDELBERG UNIVERSITY HOSPITAL]

Room B08 - B09

16:10 - 16:40 COFFEE BREAK

16:45 - 18:45

5: Cancer immuno – gene therapy

Chairs David Klatzman, Helen Heslop

INV70 Adoptive T cell therapy
Thomas Blankenstein [CHARITÉ MEDICAL UNIVERSITY BERLIN]

INV71 Harnessing Sleeping Beauty for T-cell therapies: From bench to bedside to boardroom
Laurence Cooper [MD ANDERSON CANCER CENTRE, ZIOPHARM, BOSTON, MA]

INV72 Individualised cancer immunotherapy
Ugur Sahin [JOHANNES GUTENBERG UNIVERSITY, MAINZ]

INV73 Axicabtagene ciloleucel, an anti-CD19 CAR T-cell therapy for B-cell leukaemia and lymphoma
Zachary Roberts [KITE PHARMA, LOS ANGELES, CA]

Room C01

18:45 - 20:15 : Poster session 2 (Please see page 103)

20:30 MOLECULAR MINGLE EVENING (SEE PAGE 12)

Programme Friday 20 October 2017

09:00 - 10:30

5a: Gene editing II

Chairs Hildegard Büning, Keith Joung

INV74 **Targeted genome editing in haematopoietic stem/progenitor cells for the treatment of inherited diseases**
Pietro Genovese [HSR TIGET, MILAN]

INV75 **New zinc finger nuclease architectures for precision genome engineering in the development of novel genomic therapie**
Michael Holmes [SANGAMO THERAPEUTICS, RICHMOND, CA]

OR40 **A novel designer epigenome modifier platform enables robust and sustained gene silencing in clinically relevant human cells**
Claudio Mussolino [UNIVERSITY OF FREIBURG]

OR41 **In trans paired nicking triggers seamless genome editing without double-stranded DNA cutting**
Xiaoyu Chen [LEIDEN UNIVERSITY MEDICAL CENTRE]

Room C01

5b: Cancer gene therapy II

Chairs Ramon Alemany, Wolfgang Uckert

INV76 **Engineering of CAR-T cells with enhanced therapeutic index using virus-free gene-transfer and genome-editing strategies**
Michael Hudecek [UNIVERSITY CLINIC, WÜRZBURG]

INV77 **Use of a telomerase reactivation peptide (GSE4) to treat rare diseases with low telomerase activity**
Rosario Perona [CSIC-UAM, MADRID]

Room B05 - B07

Programme Friday 20 October 2017

09:00 - 10:30

5b: Cancer gene therapy II continued

OR42 **Immunotherapy for tumours through APOBEC3B-induced neo-epitope generation in combination with immune checkpoint blockade**
Richard Vile [MAYO CLINIC, ROCHESTER, MN]

OR43 **CRISPR-CAR vector couples transgene expression to target scission for generation of universal T cells**
Roland Preece [UNIVERSITY COLLEGE LONDON]

Room B05 - B07

5c: Non-viral gene therapy

Chairs Zoltan Ivics, Seppo Ylä-Herttuala

INV78 **Transposon-based, targeted *ex vivo* gene therapy to treat age-related macular degeneration – a summary of the pre-clinical data**
Gabriele Thumann [UNIVERSITY OF GENEVA]

INV79 **Engineering the genome with the Sleeping Beauty transposon system**
Zoltan Ivics [PAUL EHRLICH INSTITUTE, LANGEN]

OR44 **Engineered exosomes for non-viral delivery of RNA therapeutics to brain**
Reka Haraszti [UNIVERSITY OF MASSACHUSETTS MEDICAL SCHOOL, WORCESTER, MA]

OR45 **Reversible immortalisation allows genetic correction of human skeletal muscle progenitors and generation of novel human artificial chromosomes for Duchenne muscular dystrophy**
Sara Benedetti [UNIVERSITY COLLEGE LONDON]

Room B08 - B09

10:30 - 11:00 COFFEE BREAK

Main Congress

INV Invited speaker • OR Selected abstracts • P Lightening talks

Programme Friday 20 October 2017

11:00 - 13:00

6: New tools and technologies: Gene and genome editing and engineering

Room C01

Chairs Luigi Naldini, Toni Cathomen

INV80 Efficient gene editing and gene regulation using CRISPR-Cpf1 nuclease technology
Keith Joung [MASSACHUSETTS GENERAL HOSPITAL, BOSTON, MA]

INV81 Programmable nucleases and designer recombinases for genome surgery
Frank Buchholz [TECHNICAL UNIVERSITY DRESDEN]

INV82 Targeting non-coding RNAs for tissue repair and regeneration
Stefanie Dimmeler [GOETHE UNIVERSITY FRANKFURT AM MAIN]

INV83 Cerebral organoids: Modelling human brain development and tumourigenesis in stem cell derived 3D culture
Juergen Knoblich [INSTITUTE OF MOLECULAR BIOTECHNOLOGY GMBH, VIENNA]

13:00 - 14:30 LUNCH

14:30 - 17:00

Presidential symposium, AGM and awards ceremony

Room C01

Chairs Robin Ali, Zoltan Ivics

Presidential talk and AGM
Robin Ali [UNIVERSITY COLLEGE LONDON]

INV84 Building synthetic chromosomes from scratch
Jef Boeke [NEW YORK UNIVERSITY]

INV85 Human Gene Therapy — Outstanding Achievement Award
T cell immunotherapy: Practice makes perfect?
Malcolm Brenner
[BAYLOR COLLEGE OF MEDICINE, HOUSTON, TX]

OR46 Molecular Therapy Family of Journals — Young Investigator Award
Engineering complex genotypes in primary haematopoietic cells using Cas9/sgRNA and AAV donor vectors
Rasmus Bak [STANFORD UNIVERSITY, CA]

Notes: