EDUCATION SESSION

TUESDAY 16 OCTOBER

09:15-09:30
GARDEN FLOOR: CONFERENCE ROOM 2/3

Organiser and Chair:
Hildegard Büning  Hannover Medical School

EDUC 1: OPENING WORDS
INV013: Hildegard Büning  Hannover Medical School
Gene and cell therapy - a brief update

09:30-11:00
GARDEN FLOOR: CONFERENCE ROOM 2/3

EDUC 2: TECH. SPEED DATING
(15 minutes each and 15 for questions)

INV014: Els Verhoeyen  CRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice
Novel lentiviral pseudotypes for natural killer based cancer immunotherapies and ‘nanoblades’ for efficient gene editing in T, B, IPS cells and blood stem cells

INV015: Karim Benihoud  CNRS UMR 8203, University Paris-Sud, Villejuif
INV013: Hildegard Büning  Hannover Medical School
Insights into adenovirus and AAV vectorology

INV016: Eduard Ayuso  INSERM UMR1089, University of Nantes
Manufacturing and quality control of viral vectors

INV017: Claudio Mussolino  University of Freiburg
How to re-write or epigenetic control our genetic information

INV018: Michel Pucéat  INSERM U1251, Marseille
A brief overview on iPSC and embryonic stem cell technology

11:00-11:30
CAMPUS FLOOR

Coffee break

11:30-12:30
GARDEN FLOOR: CONFERENCE ROOM 2/3

EDUC 3: CANCER – WHERE DO WE STAND AND HOW TO MOVE FORWARD?

INV019: Vincenzo Cerullo  University of Helsinki
Dressing viruses in tumor’s clothing: welcome to the cloning-free oncolytic vaccine era

INV021: Delphine Fessart  INSERM U913, University of Bordeaux
Cancer stem cells and organoids development: towards a better understanding of the biology behind organoids

12:30-13:15
CAMPUS FLOOR

Lunch

13:15-14:45
EDUC 4: CNS SESSION

INV021: Nicole Déglon  Lausanne University Hospital
Introduction to gene and cell therapy in the CNS

INV022: Nathalie Cartier  INSERM/ CEA UMR1169, MIRCEN CEA and University Paris-Sud, University Paris Saclay
Gene therapy for Huntington’s disease

INV023: Ron Crystal  Weill Cornell Medical College, New York City, NY
Gene therapy for Alzheimer’s disease

14:45-16:45
EDUC 5: HSC STEM CELLS - WHERE DO WE STAND AND HOW TO MOVE FORWARD

INV025: Adrian Thrasher  University College London Institute of Child Health
Primary immunodeficiency, gene therapy vs. alternative strategies how do we move into the “real” clinical reality

INV026: Juan Bueren  CIEMAT/CIBERER-ISCIII, Madrid
Gene therapy in bone marrow failure syndromes

INV027: Olivier Nègre  BlueBirdBio, Cambridge, MA
Haematopoietic stem cell gene therapy

INV020: Luigi Naldini  SR-TIGET, Milan
HSC gene therapy: from lentiviral gene transfer to gene editing

16:45-17:00
CAMPUS FLOOR

Coffee break
# Clinical Trial and Commercialisation Workshop

**Tuesday 16 October**

**Organisers:**
- Alessandro Aiuti, SR-TIGET, Milan

<table>
<thead>
<tr>
<th>Time</th>
<th>Location</th>
<th>Session Title</th>
<th>Speaker(s)</th>
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</table>
| 9:30-11:15    | Cloud FLOOR: Auditorium C | Planning and Running a Clinical Trial | **INV001**: Kim Champion  
University College London Clinical Trials Centre  
General considerations for setting up a clinical trial  
**INV002**: Paola Albertini  
SR-TIGET, Milan  
Quality requirements for GLP tox testing and GCLP clinical testing  
**INV003**: Marco Anelli  
ProductLife Group, Milan  
Pharmacovigilance from a sponsor, CRO point of view  
**INV004**: Chiara Bonini  
SR-TIGET, Milan  
Registry platform for gene and cell therapy: the EBMT approach |
| 11:15-11:45   | Campus FLOOR | Coffee | |
| 11:45-13:15   | Cloud FLOOR: Auditorium C | Manufacturing of Gene and Cell Products | **INV005**: Jean-François Brunet  
Lausanne University Hospital  
Building academic GMP facility  
**INV006**: Xin Swanson  
Lonza, Houston, TX  Scaling GMP AAV production  
**INV007**: James Miskin  
Oxford Biomedica  Scaling GMP lentiviral vector production |
| 16:30-17:00   | Campus FLOOR | Coffee | |
PROGRAMME

TUESDAY 16 OCTOBER

17:00-19:00
CAMPUS FLOOR: AUDITORIUM A
2018 OPENING SESSION
Chairs:
Robin Ali, Pierre Cordelier, Nicole Deglon, Nancy Witty

INV028: Grégoire Courtine
EPFL, Lausanne
Targeted neurotechnologies enabling walking after paralysis

INV029: Didier Trono (2018 Outstanding Achievement Award lecture)
EPFL, Lausanne
Retroelements, their polydactyl controllers and the specificity of human biology

19:00-20:00
CAMPUS FLOOR
WELCOME RECEPTION

WEDNESDAY 17 OCTOBER

09:00-10:40
GARDEN FLOOR: CONFERENCE ROOM 2/3
S1A: DISEASE MODELLING
Chair:
Amy Wagers

INV030: Eva Hedlund
Karolinska Institute, Stockholm
Elucidating early disease mechanisms in ALS using stem cells

INV031: Holger Willenbring
University California San Francisco, CA
A mouse model of a human cholestatic liver disease reveals extent and therapeutic potential of mammalian transdifferentiation

09:00-10:40
CAMPUS FLOOR: AUDITORIUM A
SESSION 1B: CANCER GENE AND CELL THERAPY
Chairs:
Vincenzo Cerullo, Nicolas Boisgérault

INV032: Waseem Qasim
University College London
Genome engineered T cell immunotherapies for leukaemia

INV033: Kah Whye Peng
Mayo Clinic, Rochester, MN
VSV-IFNb-NIS, an armed and trackable oncolytic vesicular stomatitis virus
PROGRAMME

WEDNESDAY 17 OCTOBER

09:00-10:40
CAMPUS FLOOR:
AUDITORIUM A

Proffered papers:
OR003: Micaela Harrasser
University College London
Effective targeting of ROR1+ solid tumours with next-generation chimeric antigen receptor therapy
OR004: Catia Traversari
MolMed S.p.A., Milan
In vivo antitumour activity of a hCD44v6-specific chimeric antigen receptor in syngeneic models of solid tumours

Presented posters:
P093: Chin Yan Chang
Osaka University
RIG-I pathway stimulation polarizes neutrophils to anti-tumor type neutrophil and suppress tumor growth
P019: Silke Uhrig-Schmidt
University Hospital Heidelberg
CARAAVs – a novel class of CAR-antagonists in cancer immunotherapy
P328: Razieh Monjezi
University Hospital Würzburg
CRISPR/Cas9 unites with Sleeping Beauty to generate CAR-T cells with enhanced therapeutic index for fighting against immunosuppressive tumour microenvironment

09:00-10:40
CLOUD FLOOR:
AUDITORIUM C

SESSION 1C: CNS AND EYE DISEASES
GENE AND CELL THERAPY I

Chairs: Alberto Auricchio, Yvan Arsenijevic

INV034: Ian MacDonald
University of Alberta, Edmonton, AB
Gene therapy for choroideremia: what have we learned from the clinical trials to date

INV088: Alberto Auricchio
TIGEM, Naples
Expanding AAV transfer capacity in the retina

10:40-11:10
Coffee break

11:10-13:10
CAMPUS FLOOR:
AUDITORIUM A

PLENARY SESSION 2: FROM BENCH TO BEDSIDE I

Chair: Luigi Naldini

INV036: Stuart Forbes
Edinburgh University, Centre of regenerative medicine
Macrophage therapy for liver disease – preclinical and clinical

INV037: Amy Wagers
Harvard University, Cambridge, MA
In vivo gene editing in tissues and tissue stem cells

Travel grant awarded by:

Proffered papers:
OR005: Samiah Al Zaidy
Centre for Gene Therapy, Nationwide Children’s Hospital, Columbus, OH
AVXS-101 phase 1 gene replacement therapy clinical trial in spinal muscular atrophy type 1 (SMA1): 24-month event-free survival and achievement of developmental milestones
OR006: Stylianos Michalakis
Ludwig Maximilian University, Munich
Gene supplementation therapy for CNGA3-linked achromatopsia

Presented posters:
P222: Rui Nobre
University of Coimbra
Non-invasive allele-specific silencing therapy and biomarkers for Machado-Joseph Disease
P223: Ruslan Grishanin
Adverum Biotechnologies, Menlo Park, CA
Long-term aflibercept expression levels in non-human primates following intravitreal administration of ADVM-022, a potential gene therapy for wet age-related macular degeneration
WEDNESDAY 17 OCTOBER

11:10-13:10

INV038: Bev Davidson  
The Children's Hospital of Philadelphia and The University of Pennsylvania  
Emerging therapies for neurodegenerative diseases

INV039: Wing Yen Wong  
BioMarin, Novato, CA  
Gene therapy in haemophilia: from vision to reality

13:10-15:10

Lunch

POSTER SESSION I : ODD NUMBERS (see p116)

13:10-13:30

INV040: Fiona Watt  
King's College, London  
Identifying extrinsic and intrinsic drivers of variation in cell behaviour in human iPS cell lines

INV041: Kevin Eggan  
Harvard University, Cambridge, MA  
Reducing noise and bias from studies of disease-implicated genetic variation through massively-mosaic stem cell systems

INV091: Tenneille Ludwig  
WiCell, Madison WI  
Identification of recurrent genetic variants in hPSCs; a changing landscape

13:45-14:45

SFTCG AGM

REGULATORY WORKSHOP

GARDEN FLOOR: CONFERENCE ROOM 2/3  
Alison Armstrong

15:10-16:50

SESSION 2A: GENOMIC CHARACTERISATION OF PLURIPOTENT STEM CELLS

Chair: Ludovic Vallier

INV040: Fiona Watt  
King's College, London  
Identifying extrinsic and intrinsic drivers of variation in cell behaviour in human iPS cell lines

INV041: Kevin Eggan  
Harvard University, Cambridge, MA  
Reducing noise and bias from studies of disease-implicated genetic variation through massively-mosaic stem cell systems

INV091: Tenneille Ludwig  
WiCell, Madison WI  
Identification of recurrent genetic variants in hPSCs; a changing landscape

SESSION 2B: VECTOR DEVELOPMENT I

Chairs:  
Els Verhoeyen, Axel Schambach

INV043: Jay Chiorini  
MPTB, NIDCR, NIH, Bethesda, MD  
Characterisation of AAV44.9

INV044: Dirk Grimm  
Bioquant, Heidelberg  
Small but increasingly mighty - latest advances in AAV biology and vector optimization

Proffered papers:

OR007: Kleopatra Rapti  
Heidelberg University Hospital  
Generation of novel immune-evading AAVs through identification and mutation of immunogenic epitopes in the variable capsid regions of adeno-associated virus 9

OR008: Monica Volpin  
SR-TIGET, Milan  
The impact of vector integration on chromatin architecture

Presented posters:

P483: Weihe Song  
University of Oxford  
Exploiting adenosine mechanisms for the enhanced production of AAV vectors

P484: Jihad El Andari  
University Hospital Heidelberg  
Identification of new muscle-tropic adeno-associated virus (AAV) capsids for treatment of rare hereditary muscular disorders
WEDNESDAY 17 OCTOBER

15:10-16:50
CLOUD FLOOR: AUDITORIUM C
SESSION 2C: EMA/CAT REGULATORY ASPECTS OF ADVANCED THERAPY MEDICINAL PRODUCTS (ATMPs)

Chairs:
Martina Schüssler-Lenz, Ilona Reischl

INV045: Martina Schüssler-Lenz
CAT Chair, Paul Ehrlich Institute, Langen
Introduction to the committee for advanced therapies (CAT) and its tasks in the evaluation of advanced therapies

INV046: Marcos Timón
Medicines Agency AEMPS, Madrid
EU regulatory aspects of CAR-T cells

INV047: Hans Ovelgönne
Medicines Agency MEB, Utrecht
EU regulatory aspects of rAAV vectors

INV048: Matthias Renner
Paul Ehrlich Institute, Langen
EU regulatory aspects of genome editing

ROUND TABLE DISCUSSION
16:50-17:20
Coffee break

WEDNESDAY 17 OCTOBER

17:20-18:50
CAMPUS FLOOR: AUDITORIUM A
PLENARY SESSION 3: CANCER IMMUNOTHERAPY AND CANCER STEM CELLS

Chairs:
Chiara Bonini, Robert Blelloch

INV049: Hinrich Abken
University Hospital Regensburg
CARs and TRUCKs: next generation adoptive cell therapy

INV050: Yasuhiro Yamada
University of Tokyo
Dissecting cancer biology with iPS cell technology

INV051: Robert Blelloch
University of California, San Francisco, CA
Exosomal PD-L1 as an immune-modulator in cancer

20:00-20:30
INVITED SPEAKER DINNER (INVITATION ONLY)
Coaches will leave SwissTech at 19.00 back to the speaker dinner venue
THURSDAY 18 OCTOBER

SESSION 3A: BIOENGINEERING

Chair:
Molly Stevens

INV052: David Schaffer
University of California, Berkeley, CA
Molecular elucidation and engineering of stem cell fate decisions

INV053: Penney Gilbert
University of Toronto, ON
Making 3D models that matter: engineering skeletal muscle tissue in a dish

Proffered papers:

OR009: Pauline Schmit
Harvard University, Cambridge, MA
Cross-packaging control in multiplexed AAV libraries

OR010: François du Plessis
uniQure, Amsterdam
Development of a next generation synthetic promoter for liver directed gene therapy

Presented posters:

P168: Ekaterina Naumenko
Kazan Federal University
Enhanced dark-field microscopy for histological detection of nanostructured scaffolds after implantation into bone defects

P169: Albert Rizvanov
Kazan Federal University
Migration ability of human polymorphonuclear leukocytes loaded with synthetic microcapsules

THURSDAY 18 OCTOBER

SESSION 3B: MUSCLE & CARDIOVASCULAR DISEASES

Chairs:
Michel Pucéat, Uta Griesenbach

INV054: Ana Buj Bello
Genethon, Evry
Gene therapy of myotubular myopathy: from preclinical studies to a clinical trial

INV055: Silvia Priori
Istituti Clinici Scientifici Maugeri, Pavia
Gene therapy to prevent chaotic behaviours in cardiac electrophysiology

INV056: Antoine de Vries
Leiden University Medical Centre
Shining light on cardiac tachyarrhythmias

Presented posters:

P392: Capucine Trollet
Sorbonne University, Paris
BB-301: a single "silence and replace" AAV-based vector for the treatment of oculopharyngeal muscular dystrophy (OPMD)

P247: Francesco Tedesco
University College London
High-fidelity disease modelling of skeletal muscle laminopathies using LMNA-mutant human iPSC cells and bioengineered muscles
THURSDAY 18 OCTOBER

9:00-10:40  SESSION 3C: BLOOD DISORDERS I

CAMPUS FLOOR: AUDITORIUM A

Chairs:
Juan Bueren, Waseem Qasim

INV057: Marina Cavazzana
Imagine Institute, Paris
Gene therapy of hemoglobinopathies

INV058: Bernhard Gentner
SR-TIGET, Milan
Towards next-generation gene therapy with ex vivo-engineered haematopoietic stem and progenitor cells

Proffered papers:

OR011: Frank Staal
Leiden University Medical Centre
Developing stem cell-based gene therapy for RAG1-deficient-SCID

OR012: Paula Rio
CIEMAT-CIBERER-IIS/FJD, Madrid
Gene therapy trial in non-conditioned Fanconi anemia patients

Presented posters:

P052: Carlos Carrascoso
CIEMAT-CIBERER-IIS/FJD, Madrid
Towards the gene therapy of the bone marrow failure in patients with dyskeratosis congenita

P053: Pamela Quaranta
SR-TIGET, Milan
Role of peripheral blood circulating haematopoietic stem/progenitor cells during physiological hematopoietic maturation and after gene therapy

10:40-11:10  Coffee break

11:10-12:40  PLENARY SESSION 4: FROM BENCH TO BEDSIDE II

CAMPUS FLOOR: AUDITORIUM A

Chairs: Adrian Thrasher, Malin Parmar

INV059: Deepak Srivastava
Gladstone Institutes, San Francisco, CA
Cardiac development: basis for disease and regeneration

INV060: Kristin Baldwin
Scripps Research, San Diego, CA
Precision reprogramming approaches to cardiovascular and neurologic disease

INV061: Lorenz Studer
Memorial Sloan Kettering Cancer Center, New York, NY
Towards a pluripotent-based cell therapy for Parkinson’s disease

12:40-14:40  Lunch

13.15-14.15  POSTER SESSION II : EVEN NUMBERS (see p138)

GARDEN FLOOR

WORKSHOP ON THE USE OF LENTIVIRAL VECTORS FOR IN VIVO GENE THERAPY

GARDEN FLOOR: CONFERENCE ROOM 2/3

Speakers: Luigi Naldini, SR-TIGET, Milan
Liver-directed lentiviral gene therapy of hemophilia
Deborah Gill, University of Oxford
Lung-targeted SIV gene therapy for cystic Fibrosis
Kyri Mitropoulos, Oxford Biomedica
Clinical experience of local administration of lentiviral vectors in retinal disorders and Parkinson’s disease
THURSDAY 18 OCTOBER

14:40-16:50  GARDEN FLOOR: CONFERENCE ROOM 2/3
SESSION 4A: MOLECULAR BASIS OF DEVELOPMENTAL POTENTIAL
Chair: Amander Clark

INV062: Jennifer Erwin
The Lieber Institute for Brain Development, Baltimore, MD
Repetitive elements in stem cells

INV063: Ludovic Vallier
Wellcome Sanger Institute, Cambridge
Mechanisms controlling cell fate decisions in human pluripotent stem cells

Proffered papers:
OR026: Alberto De Iaco
EPFL, Lausanne
DPPA2 and DPPA4 regulate expression of Dux in mouse embryonic stem cells

THURSDAY 18 OCTOBER

14:40-16:50  CLOUD FLOOR: AUDITORIUM C
SESSION 4B: VECTOR DEVELOPMENT II
Chairs: Hildegard Bünning, Karim Benihoud

INV064: Rob Kotin
University of Massachusetts Medical School, Worcester, MA
Orthologous dependoparvovirus molecular fossils may provide a source of novel structural motifs and capsids for rAAV

INV065: Jude Samulski
University of North Carolina, Chapel Hill, NC
Gene therapy for DMD: from bench to bedside

INV066: Eduard Ayuso, University of Nantes
Starting from the end: analytics driving the manufacturing process of viral vectors

Proffered papers:
OR014: Mathieu Mevel, University of Nantes
NextGenAAV: a mix of organic chemistry and vectorology
OR015: Sonja Kleinlogel, University of Bern
Evolution of recombinant adeno-associated viral vectors for favorable retinal penetration properties

Presented posters:
P485: Pasqualine Colella
University of Paris-Saclay
Tandem promoter design confers tolerogenic and persistent transgene expression to AAV gene therapy in neonate Pompe mice

14:40-16:50  CAMPUS FLOOR: AUDITORIUM A
SESSION 4C: GENE EDITING
Chairs: Keith Joung, Paula Rio

INV067: Toni Cathomen
University of Freiburg
New insights in CRISPR/Cas specificity, DNA repair dynamics and DNA repair outcomes in gene edited human haematopoietic stem cells

INV068: Yong Chang
Intellia Therapeutics, Cambridge, MA
Delivering on the therapeutic potential of CRISPR/Cas9: development of an LNP-mediated genome editing therapeutic for the treatment of ATTR

Proffered papers:
OR016: Gerald Schwank
ETH, Zürich
Correction of autosomal recessive disorders via CRISPR-associated base editors in adult animals
OR017: Jae Young Lee
ToolGen, Seoul
CRISPR/Cas9-mediated downregulation of PMP22 ameliorates Charcot-Marie-Tooth disease 1A in mice
OR018: Jonathan Finn
Intellia Therapeutics, Cambridge, MA
Supra-therapeutic levels of transgene expression achieved in vivo by CRISPR/Cas9 mediated targeted gene insertion
OR019: Anais Amaya
The University of Sydney, NSW
Successful in vivo editing of patient-derived primary human hepatocytes
THURSDAY 18 OCTOBER

Presented posters:
P298: Alessia De Caneva
ICGEB, Trieste
Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases
P299: Antonio Casini
University of Trento
evoCas9, a highly specific SpCas9 variant from a yeast in vivo screening
P300: Els Verhoeven
CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice
Efficient genome editing in primary human T, B and HSCs using Baboon envelope gp pseudotyped virus derived "Na-noblades" loaded with Cas9/gRNA ribonucleoproteins

Thursday 18 October

16:50-17:20
Coffee break

17:20-19:20
PLENARY SESSION 5: NEW TOOLS AND TECHNOLOGIES

Chairs: Robin Ali, Gordon Keller

INV069: Keith Joung
Harvard Medical School, Cambridge, MA
In vivo CRISPR gene editing with no detectable genome-wide off-target mutations

INV070: David Russell
University of Washington and Universal Cells, Seattle, WA
Universal donor stem cells

INV71: Botond Roska
Friedrich Miescher Institute for Biomedical Research, Basel
The human retina and its organoids at single cell resolution

INV072: Alex Meissner
Max Planck Institute for Molecular Genetics, Berlin
Differential regulation of Oct4 targets facilitates reacquisition of pluripotency

Friday 19 October

09:00-10:40
SESSION 5A: CELL THERAPY AND REPLACEMENT

Chair: Stuart Forbes

INV073: Asuka Morizane
University of Kyoto
Cell therapy for Parkinson’s disease with induced pluripotent stem cells

INV074: Hanna Mikkola
University of California, Los Angeles, CA
MLLT3 governs human hematopoietic stem cell self-renewal

Proffered papers:
OR020: Andrea Schejtman
University College London
Towards clinical application of a lentiviral gene therapy protocol for p47phox deficient chronic granulomatous disease

OR021: Ilaria Meloni
University of Siena
Toward gene editing in Rett syndrome

Presented posters:
P170: Immacolata Brigida
SR-TIGET, Milan
Gene therapy for adenosine deaminase 2 deficiency
FRIDAY 19 OCTOBER
09:00-10:40
CAMPUS FLOOR: AUDITORIUM A

SESSION 5B: METABOLIC AND LYOSOMAL DISEASES

Chairs:
Federico Mingozzi, Fatima Bosch

INV075: Juan Ruiz
Abeona Therapeutics, Dallas, TX
Treatment of lysosomal storage diseases (MPS-III A and III B) by intravenous administration of AAV vectors

INV076: Michel Zerah
Necker Hospital, Paris
Intracerebral gene therapy: neurosurgical point of view

Proffered papers:
OR023: Brian Bigger
University of Manchester
Brain targeted stem cell gene therapy corrects mucopolysaccharidosis type II via multiple mechanisms?
OR022: Joseph Lillegard
Mayo Clinic, Rochester, MN
In utero liver-directed lentiviral gene therapy cures a pig model of hereditary tyrosinemia type 1

Presented posters:
P357: Sem Aronson
University of Amsterdam
Brain targeted stem cell gene therapy corrects mucopolysaccharidosis type II via multiple mechanisms

ROUND TABLE

10:40-11:10
Coffee break

Travel grant awarded by:
FRIDAY 19 OCTOBER

11:10-13:10

PLENARY SESSION 6: ORGANOIDS AND CELL ENGINEERING

Chairs: Robin Ali, Deepak Srivastava

INV079: Molly Stevens
Imperial College London
Exploring and engineering the cell-material interface for regenerative medicine and mechanobiology

INV080: Rick Livesey
University of Cambridge
Engineering stem cell-derived human neural tissues to study brain development and disease

INV081: Hiromitsu Nakauchi
University of Tokyo
From cells to organs: exploiting the organ niche for interspecies organogenesis

INV082: Gordon Keller
University of Toronto
Translating human development to new therapies with pluripotent stem cells

13:10-15:00

Lunch

POSTER SESSION III: ALL POSTERS

GARDEN FLOOR

13.45-14.45

EARLY CAREER RESEARCHERS CAREER’S WORKSHOP

GARDEN FLOOR: CONFERENCE ROOM 2/3

Vincenzo Cerullo, University of Helsinki
Deniz Kirik, Lund University

15:00-16:30

SESSION 6A: REGULATION AND CONTROL OF PLURIPOTENCY AND LINEAGE SPECIFICATION?

Chair: Teneille Ludwig

INV083: Keisuke Kaji
Edinburgh University
Molecular mechanisms of cellular reprogramming

INV084: Amander Clark
University of California Los Angeles, CA
Unique control of naïve pluripotency in human stem cells and the germline

Proffered papers:
OR013: Julien Pontis
EPFL, Lausanne
Evolutionarily recent transposable elements and their controllers regulate human early embryonic transcriptional network

Presented posters:
P371: Pavel Makarevich
Moscow State University
Role of paracrine factors secreted by mesenchymal stromal cells in spermatogonial stem cell niche regulation

13.45-14.45

GENE THERAPY FOR THE 21ST CENTURY PATIENT

CLOUD FLOOR AUDITORIUM

Chairs:
Nathalie Cartier, MIRCen, INSERM U986, CEA, Fontenay aux Roses
Samantha Parker, Lysogene, Paris

Speakers:
Annie Hubert, Alliance for Regenerative Medicine (ARM)
Nicole Boice, Global Genes
Cara O’Neill, Cure Sanfilippo Foundation
Elin Haf Davies, Aparito
FRIDAY 19 OCTOBER

15:00-16:30 CLOUD FLOOR: AUDITORIUM C
SESSION 6B: IMMUNE RESPONSES FOLLOWING GENE THERAPY: FROM VACCINES TO VECTOR OPTIMISATION
Chairs: Anne Galy, Jude Samulski

INV085: Maria Croyle
University of Texas, Austin
Reaching beyond the cold chain: formulation design of vaccines to improve potency, enhance distribution and modulate other biological processes

INV086: Ying Kai Chan
Harvard University, Cambridge, MA
Engineering AAV vectors to evade innate immune and inflammatory responses

Proffered papers:
OR027: Dimitrios Laurin Wagner
Charité, Berlin
T cell immunity towards CRISPR-associated nucleases
OR028: Tim Beissert
TRON, Mainz
Alphaviral trans-replicating RNA is a low dose vaccine vector

15:00-16:30 CAMPUS FLOOR: AUDITORIUM A
SESSION 6C: CNS AND EYE DISEASES II
Chairs: Nathalie Cartier, Nicole Deglon

INV087: Shin-Ichi Muramatsu
Jichi Medical University
Gene therapy for Parkinson’s disease, implications from a clinical study of AADC deficiency

INV035: Sandro Alves
Brainvectis, Paris
CYP46A1-gene therapy alleviates spinocerebellar ataxia in mouse models

Proffered papers:
OR029: Vania Broccoli
San Raffaele Hospital, Milan
Modeling functional and dysfunctional brain circuits with human iPSC-derived neurons in microfluidic chambers
OR030: Lee Ni-Chung
National Taiwan University Hospital, Taipei
Gene therapy for AADC deficiency results in de novo dopamine production and supports durable improvement in major motor milestones

16:30-17:00 Coffee break

17:00-19:00 CAMPUS FLOOR: AUDITORIUM A
PRESIDENTIAL SYMPOSIUM
ESGCT AGM
Chairs: Robin Ali, Pierre Cordelier, Nicole Deglon, Nancy Witty

INV089: Juergen Knoblich
Institute of Molecular Biotechnology, Vienna
Cerebral organoids: modelling human brain development and tumorigenesis in stem cell derived 3D culture

INV090: Rudolf Jaenisch
MIT, Cambridge, MA
Epigenetic regulation in development, ageing and disease

Proffered papers:
OR031: Alessio Cantore (Young Investigator Award lecture)
SR-TIGET, Milan
Shielding lentiviral vectors from phagocytosis increases hepatocyte gene transfer in non-human primates

19:00-19:30 CLOSING DRINKS