

# 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 Verhoeven

*CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice*  
Retro- and lentiviral vector technology

### INV015: Karim Benihoud

*CNRS UMR 8203, University Paris-Sud, Villejuif*

### INV013: Hildegard Büning

*Hannover Medical School*

Novel lentiviral pseudotypes for natural killer based cancer immunotherapies and 'nanoblades' for efficient gene editing in T, B, iPSC cells and blood stem cells

### 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

# EDUCATION SESSION

TUESDAY 16 OCTOBER

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

### INV020: Luigi Naldini

*SR-TIGET, Milan*

Lentiviral vectors: the first 20 years

### INV021: Delphine Fessart

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

Lunch

12:30-13:30

CAMPUS FLOOR

13:30-15:00

## 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

15:00-16:30

## 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-ISCI, Madrid*

Gene therapy in bone marrow failure syndromes

**INV027: Olivier Nègre** *BlueBirdBio, Cambridge, MA*

Haematopoietic stem cell gene therapy

16:30-17:00

CAMPUS FLOOR

Coffee break

# CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP

TUESDAY 16 OCTOBER

**Organisers:**

Alessandro Aiuti *SR-TIGET, Milan*

Robin Ali *UCL, Institute of Ophthalmology, London*

9:30-11:15

**PLANNING AND RUNNING A CLINICAL TRIAL**

CLOUD FLOOR:  
AUDITORIUM C

**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

Coffee

CAMPUS FLOOR

11:45-13:15

**MANUFACTURING OF GENE AND CELL  
PRODUCTS**

CLOUD FLOOR:  
AUDITORIUM C

**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

# CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP

TUESDAY 16 OCTOBER

13:15-14:15

Lunch

14:15-16:30

**PATIENT ACCESS, REGULATORY AND  
REIMBURSEMENT CHALLENGES**

CLOUD FLOOR:  
AUDITORIUM C

**INV008: Ron Philip**

*Spark Therapeutics, Philadelphia, PA*

Patient access worldwide

**INV009: Ilona Reischl**

*Federal Office for Safety in Health Care, Vienna*

Update on new ATMP guidelines for GMP production: focus on the risk based approach

**INV010: Martina Schüssler-Lenz**

*CAT Chair, Paul Ehrlich Institute, Langen*

Risk management planning in the development of ATMP

**INV011: Michela Gabaldo**

*Telethon, Milan*

Market access for ATMP in Europe

**INV012: Eric Auger**

*Putnam Associates, Boston, MA*

Market access for ATMP in US

16:30-17:00

Coffee

CAMPUS FLOOR

# PROGRAMME

## TUESDAY 16 OCTOBER

17:00-19:00

### 2018 OPENING SESSION

CAMPUS FLOOR:  
AUDITORIUM A

#### 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

### WELCOME RECEPTION

CAMPUS FLOOR

## WEDNESDAY 17 OCTOBER

09:00-10:40

### S1A: DISEASE MODELLING

GARDEN FLOOR:  
CONFERENCE  
ROOM 2/3

#### 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

# PROGRAMME

## WEDNESDAY 17 OCTOBER

### Proffered papers:

#### OR001: Jamal Alzubi

Institute for Transfusion Medicine and Gene Therapy, Freiburg

A novel chimeric antigen receptor (CAR) T cell approach eliminates prostate cancer in a mouse tumor model

#### OR002: Saskia König

Institute for Transfusion Medicine and Gene Therapy, Freiburg

Allele-specific editing of STAT3 mutations with CRISPR/Cas in primary cells of hyper-IgE syndrome patients

### Presented posters:

#### P166: Kathrin Haake

Hannover Medical School

Hematopoietic stem cell gene therapy for Ifn $\gamma$ 1 deficiency protects mice from mycobacterial infections and paves way for macrophage transplantation therapy

#### P167: Viviane Dettmer

Institute for Transfusion Medicine and Gene Therapy, Freiburg

Retroviral UNC13D transfer restores cytotoxic function in T cells derived from familial hemophagocytic lymphohistiocytosis type 3 patients

09:00-10:40

### SESSION 1B: CANCER GENE AND CELL THERAPY

CAMPUS FLOOR:  
AUDITORIUM A

#### 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-IFN $\beta$ -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

Travel grant awarded by:



### 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*

CARAAs - 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

Travel grant awarded by:



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

# PROGRAMME

## WEDNESDAY 17 OCTOBER

### 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

Coffee break

10:40-11:10

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

# PROGRAMME

## 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

CAMPUS FLOOR:  
AUDITORIUM A

**INV039: Wing Yen Wong**

*BioMarin, Novato, CA*

Gene therapy in haemophilia: from vision to reality

13:10-15:10

Lunch

GARDEN & CAMPUS  
FLOORS

**POSTER SESSION I : ODD NUMBERS** (see p116)

GARDEN FLOOR

13:10-13.30

**SFTCG AGM**

CAMPUS FLOOR: AUDITORIUM A

13:45-14.45

**REGULATORY WORKSHOP**

GARDEN FLOOR: CONFERENCE ROOM 2/3

Alison Armstrong



15:10-16:50

**SESSION 2A: GENOMIC CHARACTERISATION OF PLURIPOTENT STEM CELLS**

GARDEN FLOOR:  
CONFERENCE  
ROOM 2/3

**Chair:** Ludovic Vallier

**INV040: Fiona Watt**

*King's College, London*

Identifying extrinsic and intrinsic drivers of variation in cell behaviour in human iPSC 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



# PROGRAMME

## WEDNESDAY 17 OCTOBER

15:10-16:50

**SESSION 2B: VECTOR DEVELOPMENT I**

CAMPUS FLOOR:  
AUDITORIUM A

**Chairs:**

Els Verhoeven, 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

Travel grant awarded by:



**OR008: Monica Volpin**

*SR-TIGET, Milan*

The impact of vector integration on chromatin architecture

**Presented posters:**

**P483: Weiheng Su**

*University of Oxford*

Exploiting adenovirus mechanisms for the enhanced production of AAV vectors

Travel grant awarded by:



**P484: Jihad El Andari**

*University Hospital Heidelberg*

Identification of new muscle-tropic adeno-associated virus (AAV) capsids for treatment of rare hereditary muscular disorders

# PROGRAMME

## 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

# PROGRAMME

## 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

# PROGRAMME

## THURSDAY 18 OCTOBER

9:00-10:40

CLOUD FLOOR:  
AUDITORIUM C

### 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

# PROGRAMME

## THURSDAY 18 OCTOBER

9:00-10:40

GARDEN FLOOR:  
CONFERENCE  
ROOM 2/3

### 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 iPSCs and bioengineered muscles



# PROGRAMME

## THURSDAY 18 OCTOBER

9:00-10:40

CAMPUS FLOOR:  
AUDITORIUM A

### SESSION 3C: BLOOD DISORDERS I

#### 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

Travel grant awarded by:



10:40-11:10

Coffee break

# PROGRAMME

## THURSDAY 18 OCTOBER

11:10-12:40

CAMPUS FLOOR:  
AUDITORIUM A

### PLENARY SESSION 4: FROM BENCH TO BEDSIDE II

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

### 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 Mitrophanous, *Oxford Biomedica*

Clinical experience of local administration of lentiviral vectors in retinal disorders and Parkinson's disease



13.15-14.15



# PROGRAMME

## 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:

##### OR013: Julien Pontis, EPFL, Lausanne

Evolutionarily recent transposable elements and their controllers regulate human early embryonic transcriptional network

14:40-16:50

CLOUD FLOOR:  
AUDITORIUM C

### SESSION 4B: VECTOR DEVELOPMENT II



**Chairs:** Hildegard Büning, 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

# PROGRAMME

## THURSDAY 18 OCTOBER

#### 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

Travel grant awarded by:



# PROGRAMME

## 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 "Nanoblades" loaded with Cas9/sgRNA ribonucleoproteins

Travel grant awarded by:



16:50-17:20

Coffee break

17:20-19:20

### PLENARY SESSION 5: NEW TOOLS AND TECHNOLOGIES

CAMPUS FLOOR:  
AUDITORIUM A

**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



# PROGRAMME

## THURSDAY 18 OCTOBER

20:00

### MOLECULAR MINGLE AT THE OLYMPIC MUSEUM

Coaches will leave from SwissTech at 19.30 to the Olympic Museum



## FRIDAY 19 OCTOBER

09:00-10:40

CLOUD FLOOR:  
AUDITORIUM C

### 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*

MLL3 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

# PROGRAMME

## FRIDAY 19 OCTOBER

09:00-10:40

CAMPUS FLOOR:  
AUDITORIUM A

### SESSION 5B: METABOLIC AND LYSOSOMAL DISEASES



#### Chairs:

Federico Mingozzi, Fatima Bosch

#### INV075: Juan Ruiz

*Abeona Therapeutics, Dallas, TX*

Treatment of lysosomal storage diseases (MPS-IIIa and IIIB) 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

Travel grant awarded by:



### ROUND TABLE

# PROGRAMME

## FRIDAY 19 OCTOBER

09:00-10:40

GARDEN FLOOR:  
CONFERENCE  
ROOM 2/3

### SESSION 5C: BLOOD DISORDERS II

#### Chairs:

Olivier Nègre, Thierry VandenDriessche

#### INV077: Isabelle André-Schmutz

*Institute Imagine, Paris*

*Ex vivo* generated lymphoid progenitors for immune reconstitution in the context of allogeneic transplantation and gene therapy

#### INV078: Basil Sharrack

*University of Sheffield*

Autologous hematopoietic cell transplantation in multiple sclerosis

#### Proffered papers:

##### OR024: Elena Almarza Novoa

*CIEMAT, Madrid*

Comprehensive preclinical studies for the gene therapy of patients with leukocyte adhesion deficiency type I (LAD-I)

##### OR025: Ilana Moscatelli

*Lund University*

Haematopoietic stem cell targeted neonatal gene therapy by a clinically applicable lentiviral vector corrects osteopetrosis in oc/oc mice

#### Presented posters:

##### P054: Elena Barbon

*University of Evry*

The potential use of nanobodies delivered via AAV vectors in the treatment of haemophilia

##### P171: Cristina Olgassi

*University of Eastern of Piedmont Orientale, Novara*

Patient-specific iPSC-derived endothelial cells provide long-term phenotypic correction of haemophilia A

10:40-11:10

Coffee break

# PROGRAMME

## FRIDAY 19 OCTOBER

11:10-13:10

CAMPUS FLOOR:  
AUDITORIUM A

### PLENARY SESSION 6: ORGANOID 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*



# PROGRAMME

## FRIDAY 19 OCTOBER

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*

15:00-16:30

GARDEN FLOOR:  
CONFERENCE  
ROOM 2/3

### SESSION 6A: REGULATION AND CONTROL OF PURIPOTENCY 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:**

#### OR026: Alberto De Iaco

*EPFL, Lausanne*

DPPA2 and DPPA4 regulate expression of Dux in mouse embryonic stem cells

**Presented posters:**

#### P371: Pavel Makarevich

*Moscow State University*

Role of paracrine factors secreted by mesenchymal stromal cells in spermatogonial stem cell niche regulation

# PROGRAMME

## 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 Beisert**

*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

# PROGRAMME

## FRIDAY 19 OCTOBER

**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

Coffee break

16:30-17:00

17:00-19:00

CAMPUS FLOOR:  
AUDITORIUM A

### PRESIDENTIAL SYMPOSIUM ESGCT AGM



**Chairs:**

Robin Ali, Pierre Cordelier, Nicole Deglon, Nancy Witt

**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