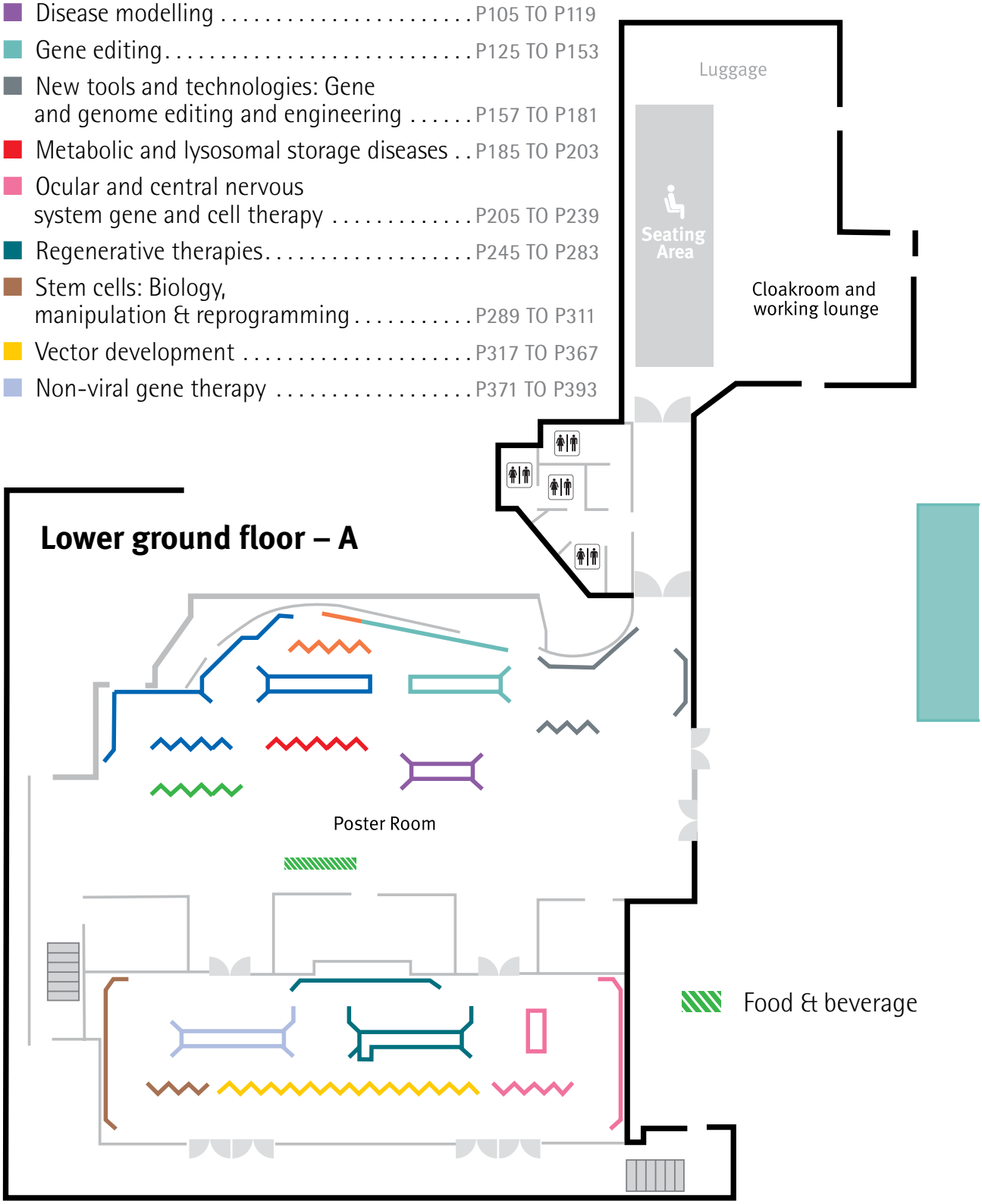
Main Congress

Poster session 1 (odd numbers) Wednesday 18 October 2017

18:30 - 21:00

- Blood disorders . . . . . P001 TO P017
- Cancer gene therapy . . . . . P021 TO P077
- Cardiovascular, muscle and pulmonary gene and cell therapy . . . . . P083 TO P099
- Disease modelling . . . . . P105 TO P119
- Gene editing . . . . . P125 TO P153
- New tools and technologies: Gene and genome editing and engineering . . . . . P157 TO P181
- Metabolic and lysosomal storage diseases . . P185 TO P203
- Ocular and central nervous system gene and cell therapy . . . . . P205 TO P239
- Regenerative therapies . . . . . P245 TO P283
- Stem cells: Biology, manipulation & reprogramming . . . . . P289 TO P311
- Vector development . . . . . P317 TO P367
- Non-viral gene therapy . . . . . P371 TO P393



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BLOOD DISORDERS

P001

V Poletti [GENETHON, INSERM UMR951, EVRY]

Pre-clinical development of a lentiviral vector expressing the anti-sickling ßAS3 globin for gene therapy for sickle-cell disease

P003

F Horling [SHIRE, VIENNA]

Development of SHP648, Shire's high performing AAV8-based FIX gene therapy vector

P005

T Ibach [HEINRICH HEINE UNIVERSITY DÜSSELDORF]

A new spacer domain from human CD34 for detection and selection of CARs

P007

S Navarro [CIEMAT/CIBERER, MADRID]

Comparative analysis of haematopoietic progenitor and stem cells in two different bone marrow failure syndromes: Diamond-Blackfan anaemia and Fanconi anaemia

P009

M Tristán-Manzano [GENYO-UNIVERSITY OF GRANADA -JUNTA DE ANDALUCIA]

Preclinical studies of an improved lentiviral vector for Wiskott-Aldrich syndrome gene therapy: Getting closer to physiological expression of WASP

P011

A Kruzik [SHIRE, VIENNA]

Assessment of biological relevance of low titer neutralising antibodies to AAV8

BLOOD DISORDERS

P013

A Kruzik [SHIRE, VIENNA]

Prevalence of pre-existing immunity to AAV8 and other AAV serotypes in healthy individuals

P015

K Cornils [UNIVERSITY MEDICAL CENTRE HAMBURG]

Adoptive transfer of gene-modified T and NK cells in a mouse model of FHL5

P017

W Hoellriegl [SHIRE, VIENNA]

Factor IX gene therapy expression in normal-weight vs. adipose haemophilia B mice

CANCER GENE THERAPY

P021

M Rovira-Rigau [IDIBAPS, BARCELONA]

Bioselection of an adenovirus encoded microRNA library identifies miR-99b and miR-485 as enhancers of adenoviral oncolysis in pancreatic cancer

P023

E Ylösmäki [UNIVERSITY OF HELSINKI]

PeptiENV: A novel cancer immunotherapy platform based on enveloped viruses coated with tumour-specific MHC-I restricted peptides

P027

T Yoshiba [JICHI MEDICAL UNIVERSITY]

Efficient targeting of uterine cervical cancer using AAV vectors encoding CRISPR/Cas9 against HPV-E6 gene

P029

J Förster [DKFZ / NCT HEIDELBERG]

Combining radiation and oncolytic measles virus for treatment of pancreatic adenocarcinoma

P031

S Feola [UNIVERSITY OF NAPLES "FEDERICO II"]

Innovative combinatorial approach in the treatment of triple negative breast cancer combining anti-PDL1 and platform based on oncolytic adenovirus (PeptiCRAd)

P033

G Halldén [BARTS CANCER INSTITUTE QMUL, LONDON]

Sensitisation to mitoxantrone-induced apoptosis by the oncolytic adenovirus AdΔΔ through Bcl-2-dependent attenuation of autophagy

P035

G Hallden [BARTS CANCER INSTITUTE QMUL, LONDON]

The novel oncolytic adenoviral mutant Ad5-3Δ-A20T retargeted to αvß6-integrins efficiently eliminates pancreatic cancer cells

P037

E Borroni [UNIVERSITA DEL PIEMONTE ORIENTALE]

Tumour targeting by lentiviral vectors combined with magnetic nanoparticles in mice

P039

S N Smith [UNIVERSITY OF ZÜRICH]

Targeted stealth adenovirus as a 'Trojan horse' for delivery of cancer therapies

P041

H Miletic [UNIVERSITY OF BERGEN]

Recurrent xenograft tumors upregulate EGFR after lentiviral vector mediated suicide gene therapy for glioblastoma, but are resistant to combinatorial treatment with erlotinib

CANCER GENE THERAPY

P043

T Takuma [MIE UNIVERSITY]

T cells expressing CAR harbouring GITR signalling domain exhibit prolonged *in vivo* survival and resistance to immunosuppression that lead to efficient tumour eradication

P045

M Doglio [SAN RAFFAELE HOSPITAL AND VITA-SALUTE SAN RAFFAELE UNIVERSITY, MILAN]

Gamma-delta CAR-T cells as an off-the-shelf cellular immunotherapy platform for paediatric B-cell lymphoblastic leukaemia

P047

H Hattori [NAGOYA MEDICAL CENTER]

Cytokine release syndrome and tumor responses in a first-in-man trial of a novel affinity-enhanced TCR-gene transduced T cell transfer targeting NY-ESO-1 antigen

P049

C E Engeland [DKFZ / NCT HEIDELBERG]

Oncolytic measles vectors for tumour-specific vaccination

P051

M A Morgan [HANNOVER MEDICAL SCHOOL]

Redirected natural killer cells to eliminate acute myeloid leukaemia cells

P053

D L Wagner [CENTER FOR CELL AND GENE THERAPY, BAYLOR COLLEGE OF MEDICINE, HOUSTON, TX]

CRISPR-mediated HPRT knockout induces chemo resistance and enables controlled depletion of gene-modified T cells

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CANCER GENE THERAPY

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J D S Hanauer [PAUL EHRLICH INSTITUTE, LANGEN]
CD30-targeted oncolytic viruses as novel therapeutic approach against Hodgkin lymphoma

P057

G Vandermeulen [CATHOLIC UNIVERSITY OF LEUVEN]
DNA vaccine coding for an engineered VSV-G as a flexible vaccination platform for cancer immunotherapy

P059

J Hartmann [PAUL EHRLICH INSTITUTE, LANGEN]
Clinical development of CAR-T cells – challenges and opportunities in translating innovative treatment concepts

P061

V I Kazey [PANACELA LABS LLC, MOSCOW]
Development of the novel adenoviral drug for treatment of prostate cancer

P063

A Lopes [CATHOLIC UNIVERSITY OF LEUVEN]
The combination of P1A DNA vaccine with immunecheckpoint blockade induces a potent antitumor immune response in P815 mastocytoma

P065

R Jofra Hernandez [HSR-TIGET, MILAN]
GLP Toxicology and tumourigenicity study for X-linked chronic granulomatous diseases in Cybb-KO mice with MSP.gp91_126T(2) LV vector

P067

C E Engeland [DKFZ / NCT HEIDELBERG]
Development of rational immunomodulation strategies for measles virotherapy

CANCER GENE THERAPY

P069

G Castellano Gonzalez [WESTMEAD INSTITUTE OF MEDICAL RESEARCH, UNIVERSITY OF SYDNEY, NEW SOUTH WALES]
Generation of a third party cryopreserved fungus-specific T lymphocytes for use in HSCT patients with invasive fungal disease

P071

F K M Lorenz [MAX DELBRÜCK CENTRE FOR MOLECULAR MEDICINE]
High efficiency T cell engineering using a novel Sleeping Beauty transposon system and minicircle vectors

P073

S M Oh [YOUSEI UNIVERSITY]
The robust viability of natural killer (NK) cells through the application of an inverted quasi-spherical (iQS) system and the t ransduction of NANOG gene

P075

S Waramit [IMPERIAL COLLEGE LONDON]
The augmentation of tumour-associated antigen expression by a hybrid bacteriophage/adeno-associated virus vector for CAR T cell therapy

P077

M Schmueck-Henneresse [CHARITÉ MEDICAL UNIVERSITY BERLIN]
Comprehensive approach for identifying the T-cell subset origin of CD3 and CD28 antibody-activated chimeric antigen receptor-modified T-cells

CARDIOVASCULAR, MUSCLE AND PULMONARY GENE AND CELL THERAPY

P083

J E L Andari [HEIDELBERG UNIVERSITY HOSPITAL]

Identification of new muscle-tropic AAV capsids for treatment of rare hereditary muscular disorders

P085

A A Titova [KAZAN FEDERAL UNIVERSITY]

The influence of intramuscular injection of genetically modified adipose-derived mesenchymal stem cells on skeletal muscle regeneration in rat hindlimb ischemia model

P087

R Blázquez [JUMISE, CÁCERES]

In vitro pro-regenerative potential of extracellular vesicles derived from CDCs

P089

J Malcher [CHARITÉ MEDICAL UNIVERSITY, BERLIN]

Exon skipping as a therapeutic strategy in dysferlinopathy

P091

R Blázquez [JUMISE, CÁCERES]

Intracoronary allogeneic cardiac progenitor cells improve myocardial salvage and enhance functional recovery compared to placebo in porcine experimental infarction

P093

R Blázquez [JUMISE, CÁCERES]

Intracoronary administration of microencapsulated IGF-1 or HGF in a swine acute infarct model: A comparative study

CARDIOVASCULAR, MUSCLE AND PULMONARY GENE AND CELL THERAPY

P095

S Y Lim [ST. VINCENT'S INSTITUTE OF MEDICAL RESEARCH, MELBOURNE, VI]

Activation of the eNOS-NO signalling pathway enhances transcription factor-mediated direct cardiac reprogramming of human fibroblasts

P097

M Pryshliak [TECHNICAL UNIVERSITY, BERLIN]

Improvement of coxsackievirus B3 (CVB3) myocarditis model by use of miR-375-regulated CVB3

P099

L Cocera Ortega [ACADEMIC MEDICAL CENTRE, AMSTERDAM]

Lentiviral vs electroporation-based gene delivery in cardiomyocyte progenitor cells to generate long-term biological pacing

DISEASE MODELLING

P105

O Humbert [FRED HUTCHINSON CANCER RESEARCH CENTRE, SEATTLE, WASHINGTON]

In vivo gene therapy for SCID-X1: A novel and accessible approach with no conditioning

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H Puccio [INSERM U596, ILLKIRCH]

Rescue of central and peripheral neurological phenotype in a novel mouse model of Friedreich's ataxia by intravenous delivery of AAV Frataxin

P109

S López-Manzaneda [CIEMAT/CIBERER, ISCIII, MADRID]

Pyruvate kinase deficient haematopoietic progenitors either from patients or generated by CRISPR/Cas9 gene editing differentiate *in vitro* along the erythroid lineage despite decreased pyruvate kinase activity

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DISEASE MODELLING

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A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Analysis of self-organisation of mesenchymal stem cells and neuroblastoma cells on Matrigel extracellular matrix

P113

E O Sahin [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

Multiple low-dose streptozotocin injections induced diabetes in wistar rats characterized by insulitis and hyperglycaemia useful for gene transfer studies

P115

G Frati [HSR-TIGET, MILAN]

Early sulfatide storage and progressive neuronal and glial cell dysfunction revealed by human iPSC-based models of metachromatic leukodystrophy are rescued by lentiviral-mediated restoration of ARSA activity

P117

A Dorronsoro [RESEARCH INSTITUTE, LA FE, VALENCIA]

Characterisation of induced pluripotent stem cells from a patient with transposition of the great arteries

P119

M N Primo [WELLCOME TRUST SANGER INSTITUTE, CAMBRIDGE]

Characterisation of type I interferon responses to viral vectors in human macrophages

GENE EDITING

P125

G Schiroli [HSR-TIGET, MILAN]

Haematopoietic stem cell gene editing for safe and effective correction of SCID-X1

GENE EDITING

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A Barzel [LOGICBIO THERAPEUTICS INC, CAMBRIDGE, MA]

GeneRide™: Therapeutic *in vivo* gene targeting without nucleases

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A Cavazza [UNIVERSITY COLLEGE LONDON]

Targeted gene correction of Wiskott-Aldrich Syndrome in human haematopoietic stem and progenitor cells

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K Stieger [JUSTUS LIEBIG UNIVERSITY, GIESSEN]

In vitro studies to investigate the therapeutic approach of replacing the defective human RPGR ORF15 gene through MMEJ

P135

B Duchêne [CENTRE DE RECHERCHE DU CHU DE QUÉBEC]

CRISPR/Cas9 induced deletion for the correction of the human dystrophin gene

P137

G Turchiano [UNIVERSITY OF FREIBURG]

Efficient GMP-compatible CRISPR-Cas mediated genome editing in haematopoietic stem and progenitor cells

P139

D Benati [UNIVERSITY OF MODENA E REGGIO EMILIA]

CRISPR/Cas9-mediated specific knock-down of dominant mutations in Rhodopsin gene

P141

H Escobar [MAX DEBRÜCK CENTRE FOR MOLECULAR MEDICINE, BERLIN]

Precise gene editing of muscular dystrophy-causing mutations in patient-derived iPS cells

GENE EDITING

P143

A Izmiryan [INSERM U1163, INSTITUT IMAGINE, PARIS]

CRISPR/Cas9-mediated COL7A1 editing in induced pluripotent stem cells from patients with recessive dystrophic epidermolysis bullosa

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L I Schwarze [UNIVERSITY MEDICAL CENTRE HAMBURG]

Addressing off-target activity of CCR5-Uco-TALEN targeting the HIV co-receptor CCR5

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D Klatt [HANNOVER MEDICAL SCHOOL]

Applying CRISPR/Cas9 for precise disease modeling of chronic granulomatous disease

P149

E V Bogoslovskaya [CENTRAL RESEARCH INSTITUTE OF EPIDEMIOLOGY, MOSCOW]

Comparison of CCR5 knockout using Cas9 and Cpf1 CRISPR nucleases

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E Grueso [PAUL EHRLICH INSTITUT, LANGEN]

CRISPR/Cas9 mediated Fanca knockout in a Sus scrofa (pig) cell line

P153

A F Hennig [CHARITÉ MEDICAL UNIVERSITY BERLIN]

Optimisation of CRISPR/Cas9-mediated knock-in of large inserts into the AAVS1 safe harbor locus

NEW TOOLS AND TECHNOLOGIES: GENE AND GENOME EDITING AND ENGINEERING

P157

Y Knopp [HANNOVER MEDICAL SCHOOL]

Alpharetroviral-MS2 chimera for efficient and transient CRISPR-Cas9 delivery

P159

A Bruhat [INRA UMR-1019, UNH, INRA DE THEIX]

Regulating the expression of therapeutic transgenes by controlled intake of dietary essential amino acids

P161

H Adams [THERMO FISHER SCIENTIFIC, WATHAM, MA]

Downstream purification solutions for viral vectors: Enabling platform approaches to advance cell and gene therapy

P163

J Wettengel [UNIVERSITY OF TÜBINGEN]

Recoding mutations by harnessing human ADARs for site-directed RNA repair

P165

C C Schmelas [HEIDELBERG UNIVERSITY HOSPITAL]

Enhanced genome editing using split Staphylococcus aureus Cas9 delivered in self-complementary Adeno-associated viral vectors

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M Luo [BLUEBIRDBIO, CAMBRIDGE, MA]

High-throughput fluorescent *in situ* hybridization (FISH) staining to determine vector copy number in a single cell

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A Nousiainen [A.I.VIRTANEN INSTITUTE, KUOPIO]

Lentivirus vector -mediated targeted integration and homologous recombination into ribosomal DNA

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NEW TOOLS AND TECHNOLOGIES: GENE AND GENOME EDITING AND ENGINEERING

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H Stepto [NATIONAL INSTITUTE OF BIOLOGICAL STANDARDS AND CONTROL, POTTERS BAR]

Development of the first WHO lentiviral vector standard: Towards the production control and standardisation of lentivirus based gene therapy products

P173

I Mancini [AMARNA THERAPEUTICS, LEIDEN]

Generation of a Vero-based packaging cell line for the production of SV40 gene delivery vector particles for use in clinical gene therapy studies

P175

I A Yakovlev [HUMAN STEM CELLS INSTITUTE, MOSCOW]

DNA and RNA editing of DYSF gene exon 26 for dysferlinopathy treatment

P177

L Jeanson-Leh [GENOSAFE, EVRY]

Quality control testing of AAV vectors, from development to assay validation

P179

A van Oorschot [XENDO B.V., LEIDEN]

CMC focus during development of vector-based gene-therapy products

P181

B Rehberger [FREELINE THERAPEUTICS, LONDON]

Manufacturing of AAV-hFIX particles in an iCELLis(®) 500 fixed-bed bioreactor meets commercial-scale requirements

OCULAR AND CENTRAL NERVOUS SYSTEM GENE AND CELL THERAPY

P185

V Calbi [IRCCS SAN RAFFAELE SCIENTIFIC INSTITUTE, MILAN]

Update on safety and efficacy of lentiviral haematopoietic stem cell gene therapy (HSC-GT) for metachromatic leukodystrophy (MLD)

P187

M Peviani [DANA-FARBER/BOSTON CHILDREN'S CANCER AND BLOOD DISORDERS CENTRE, MA]

First evidence of efficacy of allogeneic HSC transplantation in a mouse model of infantile neuronal ceroid lipofuscinosis (CLN1); pathway to promising HSC gene therapy approaches

P189

A Bosch [AUTONOMOUS UNIVERSITY OF BARCELONA]

Intrathecal AAVrh10 corrects biochemical and histological hallmarks of mucopolysaccharidosis VII mice and improves behaviour and survival

P191

M W Huston [SANGAMO THERAPEUTICS, RICHMOND, CA]

Liver-based expression of the human alpha-galactosidase A gene in a murine Fabry model results in continuous high, therapeutic levels of enzyme activity and effective substrate reduction

P193

A Calabria [HSR-TIGET, MILAN]

Molecular characterisation of haematopoietic system reconstitution in metachromatic leukodystrophy patients following haematopoietic stem cell gene therapy

P195

R J Chandler [NATIONAL INSTITUTES OF HEALTH, BERTHESDA, MD]

Anc80 and SVP Rapamycin: A novel approach to AAV gene therapy for methylmalonic acidemia

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G De Sabbata [ICGEB, TRIESTE]

Development of a novel AAV vector in combination with tolerogenic nanoparticles for the treatment of ornithine transcarbamylase deficiency

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R Ferla [TIGEM, NAPLES]

Non clinical safety and efficacy of a recombinant AAV2/8 vector administered intravenously for treatment of mucopolysaccharidosis type VI

P201

L Lagalice [ONIRIS, NANTES]

Impairment of skeletal muscle precursor in Pompe disease: A challenge for the next therapeutic strategies

P203

A D Sanlioglu [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

TRAIL protects pancreatic beta cells against the destructive effects of proinflammatory cytokines in NIT-1 cells

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N Orefice [INSERM 1169, FONTENAY-AUX-ROSES]

Optogenerapy: When implant enters the seringe era

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D Sah [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

Translation of intrathecal delivery of an AAV gene therapy targeting SOD1 for the treatment of ALS

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D Sah [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

Translation of intravenous delivery of AAV gene therapy for the treatment of CNS diseases

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S Fisson [GENETHON, INSERM, EVRY]

Anti-transgene cellular immune reponses can be induced by subretinal gene transfer with rAAV in a dose-dependent manner

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A Galy [GENSIGHT-BIOLOGICS, PARIS]

78 week follow-up study results after intravitreal rAAV2/2-ND4 (GS010) injection in patients with vision loss due to G11778A ND4 Leber Hereditary Optic Neuropathy.

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G J Ye [AGTC, ALACHA, FL]

Safety and efficacy of AAV2tYF-GRK1-hRP-GR vectors in a canine model of RPGR-XLRP

P219

M Basche [UNIVERSITY COLLEGE LONDON]

Sustained gene therapy of the corneal epithelium through AAV and lentiviral transduction of limbal epithelial stem cells

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S Alves [BRAINVECTIS THERAPEUTICS, PARIS]

AAV-CYP46A1 is beneficial in Alzheimer's disease: From mice to non-human primates

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REGENERATIVE THERAPIES

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E A Spronck [UNIQURE BIOPHARMA B.V., AMSTERDAM]

AAV5-miHTT gene therapy demonstrates sustained huntingtin lowering and functional improvement in Huntington disease mouse models

P225

C Monville [CECS I-STEM, EVRY]

Preclinical validation of a novel tissue engineered product consisting in RPE derived from human embryonic stem cells disposed on human amniotic membrane

P227

S M kleine Holthaus [UNIVERSITY COLLEGE LONDON]

Brain-directed gene therapy prolongs survival and attenuates the disease phenotype in a mouse model of neuronal ceroid lipofuscinosis

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T A Carter [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

AAV gene delivery of an anti-Tau antibody using a novel blood brain barrier penetrant capsid in wild type and P301S tauopathy mice

P231

C Bouquet [GENSIGHT-BIOLOGICS PARIS]

Ocular tolerability of AAV2.7m8-ChrimsonR-tdTomato (GS030-DP) gene therapy product on blind rd1 mice injected intravitreously and exposed to 595 nm LED light

REGENERATIVE THERAPIES

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JM Kim [CHUNG-ANG UNIVERSITY HOSPITAL]

The inhibition of microRNA 466b facilitates neurological recovery in a rat intracerebral haemorrhage model by activating insulin-like growth factor pathway

P235

A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Structural and functional features of the injured spinal cord tissue after transplantation of microglia genetically modified with GDNF

P237

F Udry [UNIVERSITY OF LAUSANNE]

Evaluation of iPS-derived retinal pigment epithelium as a model for *in vitro* lentiviral-based gene therapy

P239

M Cho [YONSEI UNIVERSITY, SEOUL]

Characterisation of AAVr3.45 variant in safety and efficacy aspects for preparing human neural stem cell-based therapeutics

STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

P245

C Scharler [PARACELSUS MEDICAL UNIVERSITY, SALZBURG]

Stepwise maturation of human iPSC into clonogenic immunosuppressive mesodermal stromal progenitors identifies key immunomodulatory pathways

P247

S Hochmann [PARACELSUS MEDICAL UNIVERSITY, SALZBURG]

No identical stromal cells: Human stromal progenitors from non-skeletal sources display limited chondrogenic potential

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S Hochmann [PARACELSUS MEDICAL UNIVERSITY, SALZBURG]

Addressing skin with IVT mRNA

P251

N Ketterl [PARACELSUS MEDICAL UNIVERSITY, SALZBURG]

A potency assay for monitoring the immunomodulatory potential of stromal cell-derived extracellular vesicles

P253

G Salguero [INSTITUTO DISTRITAL DE CIENCIA BIOETECNOLOGÍA E INNOVACIÓN EN SALUD, BOGOTA]

Engineered extracellular vesicles derived from human mesenchymal stromal cells expressing CD9, show enhanced immune supresion effects on inflammatory T cells

P255

H Y Lee [YONSEI UNIVERSITY]

Facilitated nerve regeneration by human adipose-derived stem cell-attached micropatches of heparin-based hydrogel in a sciatic nerve injury model

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J Hlavaty [UNIVERSITY OF VETERINARY MEDICINE VIENNA]

Cytokine-induced interleukin-1 receptor antagonist protein expression in genetically engineered equine mesenchymal stem cells for osteoarthritis treatment

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R P Baptista [CELL THERAPY CATAPULT, LONDON]

Development of cost efficient platforms for the industrial manufacture of pluripotent stem cell-derived products for cell therapy

STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

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P Makarevich [LOMONOSOV MOSCOW STATE UNIVERSITY]

Epicardial cardiac progenitor cell sheets results in better structural integration and neovascularisation than intramyocardial injections

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P Mancheño-Corvo [TIGENIX SAU, MADRID]

In vitro demonstration of antibacterial effect of expanded adipose stem cells (eASC)

P265

O DelaRosa [TIGENIX SAU, MADRID]

Understanding allo-sensitisation after local administration of allogeneic adipose derived stem cells (eASC)

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E A Naumenko [KAZAN FEDERAL UNIVERSITY]

Nanomodified biopolymer matrices as carriers of stem cells for the regeneration of tissue defects

P269

A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Gene activated scaffolds promote reparative osteogenesis *in vivo*

P271

P Berthet [CELLULAR DYNAMICS INTERNATIONAL, MADISON, WI]

The generation of therapeutic iPSC banks: GMP iPSC lines from common HLA haplotypes

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D A Azizova [KAZAN FEDERAL UNIVERSITY]

The use of adipose derived stromal vascular fraction in complex non-healing wounds of soft tissues and bone defects in maxillofacial surgery in dog

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STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

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G Cattaruzzi [VIVABIOCELL S.P.A., UDINE]

A novel automated, closed system solution for simple, de-risked and cost-effective autologous ATMP manufacturing: The NANT 001 bioreactor.

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H M Tasyurek [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

Glucagon like peptide-1 as an antidiabetic gene therapy agent inducing transdifferantiation of pancreatic endocrine cells

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P Martin-Duque [UNIVERSITY FRANCISCO DE VITORIA, MADRID]

The effect of PEGylated hollow gold nanoparticles on stem cell migration: Potential application in tissue regeneration

P281

J Wells [BIO-TECHNE, ABINGDON]

Development of consistent GMP-grade growth factors and cytokines for cellular therapeutics

P283

A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Structural and functional effects of adipose derived mesenchymal stem cells in a fibrin matrix application in pigs with subacute spinal cord injury

REGENERATIVE THERAPIES

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S Crippa [HSR-TIGET, MILAN]

Alterations of mesenchymal stromal cells isolated from the bone marrow of beta-thalassemia patients

REGENERATIVE THERAPIES

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D Simão [IBET/ITQB-NOVA, LISBON]

A novel *in vitro* model to decode human cardiac stem cells role in myocardial ischemia reperfusion injury

P293

I O Shum [UNIVERSITY COLLEGE LONDON]

Generation of inner ear organoids from pluripotent stem cells

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D Strunk [PARACELSUS MEDICAL UNIVERSITY, SALZBURG]

Selection of tissue factor-deficient bone marrow stromal cell transplants as a novel strategy to improve therapeutic applicability

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M Rothe [HANNOVER MEDICAL SCHOOL]

Cdkn2a-/- cells for *in vitro* genotoxicity assays

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A T El-Serafi [SHARJAH INSTITUTE FOR MEDICAL RESEARCH]

Counteracting roles of epigenetic modifiers in stem cell differentiation into the adipogenic lineage

P301

B del Río [TIGENIX SAU, MADRID]

The anti-apoptotic role of adipose mesenchymal stem cells (eASC) in PBMC

P303

P P Nimiritsky [LOMONOSOV MOSCOW STATE UNIVERSITY]

Evaluation of mechanisms underlying regenerative potential of cell sheets from human mesenchymal stromal cells

REGENERATIVE THERAPIES

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H Chung [OSONG MEDICAL INNOVATION FOUNDATION]

Quantitative tools for assessing the RT-PCR for cell therapy products

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A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Tescalcin overexpression influences mesenchymal stem cell differentiation

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A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

The effect of cisplatin and paclitaxel on ultrastructure of adipose-derived mesenchymal stem cells

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F A Khafizova [KAZAN FEDERAL UNIVERSITY]

The use of the membrane dye DiD to study migration of mesenchymal stem cells applied at the site of critical bone defect in rats

VECTOR DEVELOPMENT

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A P Dane [FREELINE THERAPEUTICS, LONDON]

Pre-clinical evaluation of an engineered AAV capsid designed to mediate higher transduction of human hepatocytes

P319

P Bouillé [FLASHCELL, TOULOUSE]

Efficient gene-editing into primary T cells using a viral RNA delivery tool based on a bacteriophage-lentivirus chimeras

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J S Boura [OXFORD BIOMEDICA]

Use of an automated cell screening system for the generation of stable HIV-1 packaging and producer cell lines for the manufacture of lentiviral vectors

VECTOR DEVELOPMENT

P323

N Laham Karam [A.I.VIRTANEN INSTITUTE, KUOPIO]

A super-enhancer mediates endothelial-specific gene expression in the context of lentiviral vectors.

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M Volpin [HSR-TIGET, MILAN]

Improving the safety of lentiviral vector integration with chromatin insulators

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F B Thalheimer [PAUL EHRLICH INSTITUT, LANGEN]

Specific gene delivery into interneurons in defined brain areas

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A Rossi [CIRI, INSERM U1111, CNRS UMR5308, LYON]

Identifying and overcoming barriers limiting AAV-mediated transduction of human dendritic cells: Effect on immune responses and gene transfer efficiency

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D Van Looveren [CATHOLIC UNIVERSITY, LEUVEN]

Engineering next generation BET-independent MLV-vectors for safer gene therapy

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C Knevelman [OXFORD BIOMEDICA]

GMP manufacturing of lentiviral vectors: Scale up considerations

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