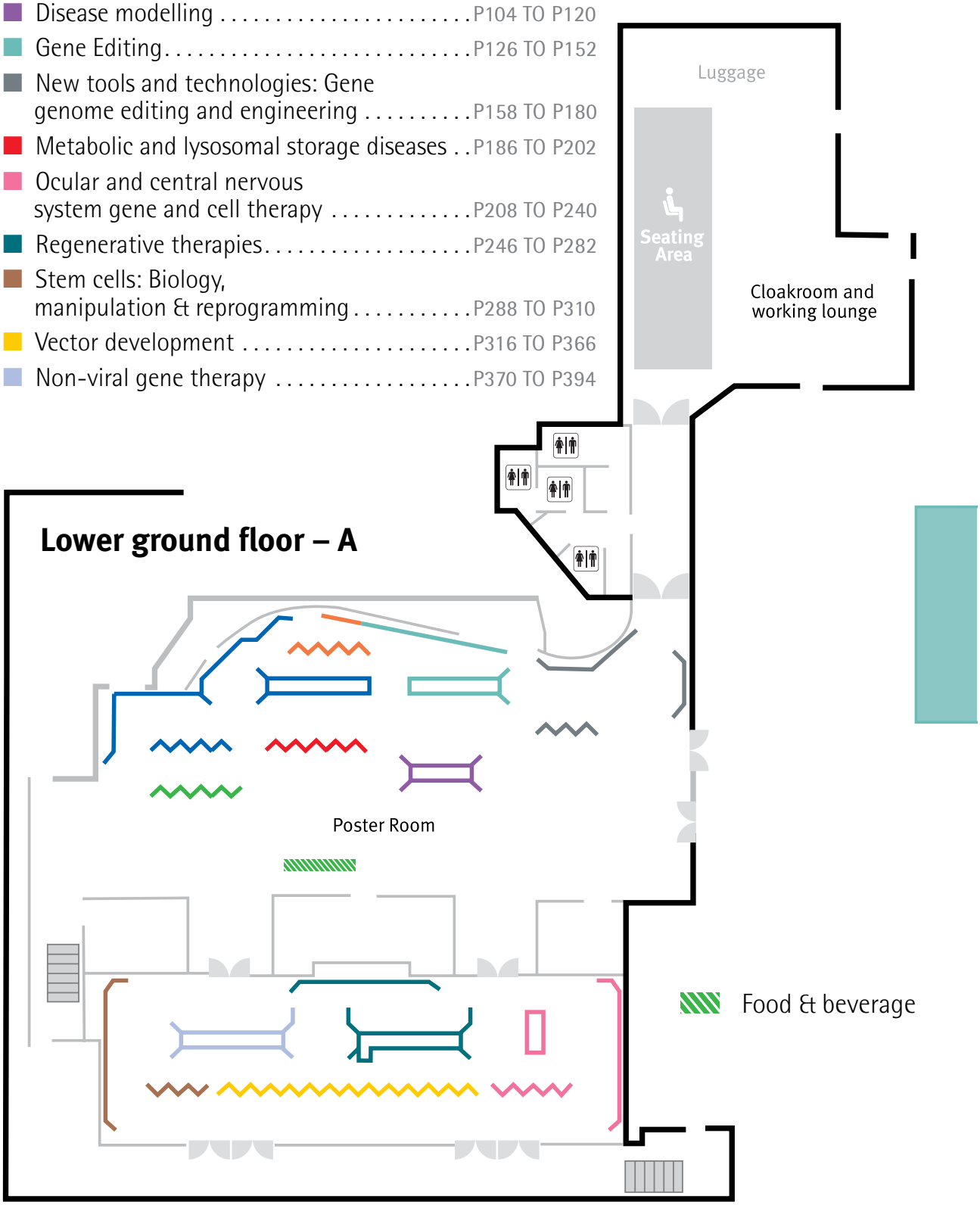
Main Congress

Poster session 2 (even numbers) Thursday 19 October 2017

18:45 - 20:15

- Blood disorders. . . . . P002 TO P018
- Cancer gene therapy. . . . . P022 TO P078
- Cardiovascular, muscle and pulmonary gene and cell therapy. . . . . P082 TO P100
- Disease modelling . . . . . P104 TO P120
- Gene Editing. . . . . P126 TO P152
- New tools and technologies: Gene genome editing and engineering . . . . . P158 TO P180
- Metabolic and lysosomal storage diseases . . P186 TO P202
- Ocular and central nervous system gene and cell therapy . . . . . P208 TO P240
- Regenerative therapies. . . . . P246 TO P282
- Stem cells: Biology, manipulation & reprogramming . . . . . P288 TO P310
- Vector development . . . . . P316 TO P366
- Non-viral gene therapy . . . . . P370 TO P394



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BLOOD DISORDERS

P002

D Cesana [HSR-TIGET, MILAN]

Expansion of human T regulatory cells by lentiviral vector mediated expression of STAT5B or BACH2 transcription factors

P004

H C Gelderblom [INTERNATIONAL AIDS VACCINE INITIATIVE, NEW YORK]

A phase 1 trial of AAV1 vectored immunoprophylaxis for HIV

P006

R Biavasco [HSR-TIGET, MILAN]

BRAFV600E expression in haematopoietic progenitors leads to senescence, myeloid skewing and aggressive histiocytosis

P008

E Verhoeyen [CIRI; INSERM U1111AND C3M INSERM U1065, NICE]

BaEV-LVs transduce HSCs-derived human progenitor T cells, allowing accelerated T cell reconstitution *in vivo* and correction of X-SCID progenitor T cells

P010

E Almarza [CIEMAT / CIBERER-ISCIII, MADRID]

Towards the gene therapy of LAD-I immunodeficient patients

P012

O Quintana-Bustamante [CIEMAT / CIBERER-ISCIII, MADRID]

Improve transduction conditions of GMP-grade lentiviral vector for pyruvate kinase deficiency gene therapy clinical trial

BLOOD DISORDERS

P014

C Mesa-Núñez [CIEMAT / CIBERER-ISCIII, MADRID]

Regulated CD18 expression in the haematopoietic gene therapy of leukocyte adhesion deficiency type I (LAD-I)

P016

J Lengler [SHIRE, VIENNA]

Correlation of *in vitro* and *in vivo* biopotency assay for FIX AAV gene therapy vectors

P018

E Payen [CEA, PARIS]

Ex-vivo selection of transduced haematopoietic stem cells for gene therapy of beta-haemoglobinopathies

CANCER GENE THERAPY

P022

A Hazini [TECHNICAL UNIVERSITY BERLIN]

MicroRNA-regulated oncolytic coxsackievirus B3 against colorectal carcinoma

P024

E Ehrke-Schulz [WITTEN/HERDECKE UNIVERSITY]

Adenoviral vectors armed with HPV oncogene specific CRISPR/Cas9 for treatment of HPV related cancers

P026

B M Pützer [ROSTOCK UNIVERSITY MEDICAL CENTRE]

Targeting E2F1-downstream pathways in invasive bladder cancer for therapeutic intervention by combining CRISPR/Cas9-mediated genome editing and adenoviral vectors

P028

C Kai [THE UNIVERSITY OF TOKYO]

Development of oncolytic measles virus, rMV-SLAMblind

P030

P Brugada-Vilà [INSTITUT QUIMIC DE SARRIA, BARCELONA]

PBAE-coated AdNuPARmE1A: Overcoming oncolytic virotherapy hurdles

P032

J Meyer [HANNOVER MEDICAL SCHOOL]

Efficient killing of target cells *in vitro* by transiently engineered chimeric antigen receptor expressing natural killer cells

P034

M Kang [CHUNGNAM NATIONAL UNIVERSITY]

Double suicide gene therapy using a GaLV-pseudotyped semi-RRV; single-shot, single-cycle suicide gene delivery system for eradication of experimental glioblastoma model

P036

Y Kim [YONSEI UNIVERSITY]

Mutagenic analysis of an AAV promoting immune resistance and efficient gene delivery

P040

S Kubo [HYOGO COLLEGE OF MEDICINE]

Retroviral replicating vector-mediated prodrug activator gene therapy in an experimental model of human osteosarcoma

P042

Y F Mei [UMEÅ UNIVERSITY]

Replication-competent adenovirus 11p vector armed with ADP gene at E1 region significantly improved tumour-killing effect on metastatic prostate cells *in vitro* and *in vivo*

CANCER GENE THERAPY

P044

R Vile [MAYO CLINIC, ROCHESTER, MN]

Inducing sensitivity to anti-PD-1 immune checkpoint blockade in murine models of melanoma and glioma

P046

K Wisskirchen [HELMHOLTZ CENTRE MUNICH]

Hepatitis B virus-specific T cell receptors with high functional avidity redirect T cells to eliminate HBV

P048

A Pfeiffer [PAUL EHRLICH INSTITUTE, LANGEN]

In vivo generation of antigen-reactive T cells for immunotherapy

P050

T Sutlu [SABANCI UNIVERSITY]

Engineering antigen-specific Natural Killer cells via TCR gene transfer: A novel source for adoptive immunotherapy

P052

R Monjezi [UNIVERSITY OF WÜRZBURG]

Enhanced engineering of chimeric antigen receptor (CAR)-modified T Cells using non-viral sleeping beauty transposition from pFAR vectors

P054

R Monjezi [UNIVERSITY OF WÜRZBURG]

Sleeping Beauty meets CRISPR/Cas9: Engineering of CAR-T cells with enhanced therapeutic index using single-step, fully non-viral gene-transfer and genome-editing

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CANCER GENE THERAPY

P056

A Mades [PAUL EHRLICH INSTITUTE, LANGEN]

Direct delivery of Sleeping Beauty transposase protein for stable integration of chimeric antigen receptor-encoding transposons from minicircle DNA vectors in human T cells

P058

T Ito [SHIN-YAMANOTE HOSPITAL]

"Artificial neoepitope"-presenting exosomes derived from the cells genetically modified to express Mycobacterium tuberculosis antigen as cellular immunity adjuvants

P060

J P W Heidbuechel [DKFZ / NCT HEIDELBERG, HEIDELBERG]

Oncolytic virotherapy to enhance BiTE and CAR efficacy against solid tumours

P062

A Barzel [TEL-AVIV UNIVERSITY]

Engineering T Cells and B cells for immunotherapy using V(D)J recombination

P064

Y Miyahara [MIE UNIVERSITY]

A novel CAR-T therapy targeting MAGE-A4p230-239/HLA-A*02:01 complex for solid tumours

P066

T Abel [TXCELL, VALBONNE]

Designing the next generation of chimeric antigen receptors for regulatory T cell therapy through *in silico* modelling-guided single chain Fv engineering

CANCER GENE THERAPY

P068

Y Koyama [SHIN-YAMANOTE HOSPITAL]

Dendritic cell activation induced by "Artificial neoepitope"-presenting exosomes derived from the genetically modified cells

P070

A Mucci [HSR-TIGET, MILAN]

Myeloid-based immunotherapy for cancer mediated by locally expressed cytokines delivered through transplantation of genetically-engineered HSPC

P072

J Kuehle [CENTRE FOR MOLECULAR MEDICINE COLOGNE]

T cell-directed enzyme-prodrug therapy (TDEPT): CAR T cells engineered for tumour-restricted activation of cytotoxic prodrugs

P074

A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Generation of genetically engineered human mesenchymal stem cells expressing immunomodulating cytokines and tumour suppressor

P076

L Dunajová [OXFORD GENETICS LTD]

Generation of stable packaging and producer cell lines for scalable lentiviral vector production

P078

V Rossella [HSR-TIGET, MILAN]

Bone marrow aging: The mesenchymal stromal cell compartment characterisation

CARDIOVASCULAR, MUSCLE AND PULMONARY GENE AND CELL THERAPY

P082

E Ehrke-Schulz [WITTEN/HERDECKE UNIVERSITY]

Delivery of a multiplex CRISPR/Cas9 machinery for the treatment of Duchenne muscular dystrophy with one single gene deleted adenoviral vector

P084

C Figueiredo [HANNOVER MEDICAL SCHOOL]

Reducing the lung immunogenicity by silencing MHC expression during *ex vivo* perfusion

P086

L Guianvarc'h [GENETHON, EVRY]

Optimisation of the triple transfection process for potent AAV production in 200L bioreactors

P088

J Ceiler [DKFZ / NCT, HEIDELBERG]

Study of wild-type and recombinant AAV-2 integration within the mitochondrial genome

P090

O N Chernova [KAZAN FEDERAL UNIVERSITY]

Effects of Ad5-DYSF transduction on skeletal muscles of dysferlin-deficient mice

P092

A Dorronsoro [RESEARCH INSTITUE, LA FE, VALENCIA]

The combination of cell and gene therapy as new generation tool to boost MSC's immunosuppressive and tissue regeneration properties

P094

E Gicquel [GENETHON, EVRY]

AAV-mediated gene transfer of FKRP for therapy of LGMD2I

CARDIOVASCULAR, MUSCLE AND PULMONARY GENE AND CELL THERAPY

P096

K Piekarowicz [UNIVERSITY OF WROCLAW]

A muscle hybrid promoter provides specific and effective gene expression after intra-muscular and systemic delivery with AAV

P098

A M Gruntman [UNIVERSITY OF MASSACHUSETTS MEDICAL SCHOOL]

Muscle as a platform for gene replacement of serum protein deficiencies

P100

L Cócera Ortega [LEIDEN UNIVERSITY MEDICAL CENTRE]

Optimising long-term delivery of biological pacemaker target genes

DISEASE MODELLING

P104

H Ibrahim [UNIVERSITY OF HELSINKI]

Modelling of RFX6 associated neonatal diabetes using CRISPR/Cas9 edited patient iPSCs

P106

G Guenechea [CIEMAT / CIBERER-ISCIII, MADRID]

Generation and potential applications of a haematopoietic stem cell model of X-linked dyskeratosis congenita

P108

L Unrau [UNIVERSITY MEDICAL CENTRE HAMBURG]

Insertional mutagenesis assisted by oncogene expression induces liver tumour formation in immunodeficient USB mice

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DISEASE MODELLING

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

Stem cells differentiation into insulin secreting cells under the influence of the epigenetic modifiers 5-Aza-2'-Deoxycytidine and suberanilohydroxamic acid

P112

Y E Eksi [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

Development of MODY10 diabetic experimental animal model using CRISPR/Cas9 technology for insulin gene transfer studies

P114

P Berthet [CELLULAR DYNAMICS INTERNATIONAL, MADISON, WI]

Human iPSC-derived midbrain dopaminergic neurons for disease modelling and cell therapy

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P Tornabene [TIGEM, NAPLES]

Three-dimensional retinal culture to model human inherited diseases

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U Rössler [CHARITÉ MEDICAL UNIVERSITY, BERLIN]

Osteoclasts differentiated from iPS cells as a test system for gene therapeutic approaches for CLCN7-related autosomal recessive osteopetrosis

P120

Y Yun [YONSEI UNIVERSITY]

Strategy for personalised medicine based on ALS patient-derived stem cells using CRISPR/Cas9-mediated genome editing

GENE EDITING

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B C Houghton [UNIVERSITY COLLEGE LONDON, GREAT ORMOND STREET HOSPITAL]

TALEN-mediated genome editing via AAV6 donor in T cells for the treatment of X-linked lymphoproliferative disease (XLP)

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M Stefanidakis [EDITAS MEDICINE, CAMBRIDGE, MA]

Development of a subretinally delivered CEP290-specific CRISPR medicine for the treatment of Leber congenital amaurosis 10 (LCA10)

P130

J Gao [WITTEN/HERDECKE UNIVERSITY]

Single vector transportation of CRISPR/Cas9 and donor DNA for homology directed repair of canine haemophilia B reveals higher repair efficiencies than utilising two vectors

P132

T Cathomen [UNIVERSITY MEDICAL CENTRE FREIBURG]

A highly efficient and GMP-compliant protocol to manufacture CCR5-edited cells to treat HIV infection

P134

S König [INSTITUTE FOR TRANSFUSION MEDICINE AND GENE THERAPY, FREIBURG]

Allele-specific inactivation of autosomal-dominant STAT3 mutations

P136

F MOREAU-GAUDRY [INSERM U1035, BORDEAUX]

Novel protocol for highly efficient CRISPR/Cas9 mediated gene correction with reduced NHEJ rate

GENE EDITING

P138

D Bachiller [CONSEJO SUPERIOR DE INVESTIGACIONES CIENTÍFICAS (CSIC)]

Correction and functional characterisation of the p.F508del cystic fibrosis mutation in patient-derived iPS cells

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B Duchêne [CENTRE DE RECHERCHE DU CHU DE QUÉBEC]

Feldan Shuttle peptide mediated delivery of Cas9/gRNAs complexes for the correction of the dystrophin gene in the Tibialis anterior of a Rag/mdx mouse model

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J Estève [INSERM U1035, UNIVERSITY OF BORDEAUX]

Targeted gene editing for phenotypic correction of primary hyperoxaluria type 1

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T Kocher [PARACELSUS MEDICAL UNIVERSITY SALZBURG]

Highly efficient and safe CRISPR/Cas9n double nicking-mediated KRT14 repair in epidermolysis bullosa simplex keratinocytes

P146

C J Stephens [WASHINGTON UNIVERSITY IN ST. LOUIS]

CRISPR/Cas9 targeted integration prolongs gene expression *in vivo*

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M Garcia-Bravo [CIEMAT / CIBERER-ISCIII, MADRID]

Gene correction of AGXT locus in a set of primary hyperoxaluria type 1 patients assisted by CRISPR/Cas9 system

GENE EDITING

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S Sánchez-Hernández [GENYO - UNIVERSITY OF GRANADA - JUNTA DE ANDALUCIA]

Comparison of ZFNs versus CRISPR specific nucleases for genome edition of the Wiskott-Aldrich syndrome locus

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F Erendor [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

Generation of a lentiviral vector encoding vasoactive intestinal peptide for type 1 diabetes

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T Pesch [ETH ZÜRICH]

In vivo lineage tracing of adoptively transferred lymphocytes using genetically encoded cellular barcodes

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J Liefhebber [UNIQURE BIOPHARMA B.V., AMSTERDAM]

Development and characterisation of MFP-inducible GeneSwitch system for AAV5 gene therapy of chronic diseases in the liver

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D Seimetz [BIOPHARMA EXCELLENCE, MUNICH]

What can we learn from case studies to address development and approval challenges of ATMPs

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B Fehse [UMC HAMBURG-EPPENDORF]

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U Koller [PARACELSUS MEDICAL UNIVERSITY SALZBURG]

Enhanced genome editing using split Staphylococcus aureus Cas9 delivered in self-complementary AAV vectors

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C Domenger [HEIDELBERG UNIVERSITY HOSPITAL]

Post-transcriptional fine-tuning of AAV vector gene expression for haemophilia A gene therapy

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I M Taylor [SOLENTIM LIMITED, WIMBORNE]

A new platform for high efficiency single cell deposition with *in-situ* image verification

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Y Kanegae [JIKEI UNIVERSITY]

The novel elimination system of HBV DNA using adenovirus vector expressing Cas9 and guide RNAs

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H B Olgun [AKDENIZ UNIVERSITY CENTRE FOR GENE AND CELL THERAPY]

A practical method for production and purification of 3rd generation HIV based lentiviral vectors for *in vivo* applications

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H Hermsen [XENDO B.V., LEIDEN]

Complex bridging and comparability strategies to implement gene therapy design modifications

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K Holden [SYNTHEGO, REDWOOD CITY]

Synthetic sgRNA enables highly efficient CRISPR editing of primary cells for therapeutic applications

OCULAR AND CENTRAL NERVOUS SYSTEM GENE AND CELL THERAPY

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A Ribera [AUTONOMOUS UNIVERSITY BARCELONA-CBATEG]

Correction of visual and auditory function by AAV9-mediated gene therapy in a mouse model of mucopolysaccharidosis type IIIB

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G Ronzitti [INTEGRARE, GENETHON, INSERM, UNIV EVRY, UNIVERSITÉ PARIS-SACLAY]

Rescue of GSDIII phenotype with gene transfer requires liver- and muscle- targeted GDE expression

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R S McIvor [IMMUSOFT CORPORATION, SEATTLE, WA]

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

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B Gentner [HSR-TIGET, MILAN]

Ex vivo HSC-based gene therapy for mucopolysaccharidosis type I

P194

C T Nicolas [MAYO CLINIC, ROCHESTER, MN]

In utero correction of hereditary tyrosinemia type 1 in mice through liver-directed lentiviral gene therapy

P196

S J Aronson [TYTGAT INSTITUTE, AMSTERDAM]

Clinical screening and assessment of pre-existing immunity against AAV in patients with Crigler-Najjar syndrome

OCULAR AND CENTRAL NERVOUS SYSTEM GENE AND CELL THERAPY

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I Azario [UNIVERSITY OF MILANO-BICOCCA]

Neonatal umbilical cord blood transplantation halts disease progression in the murine model of MPS-I

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F Ornaghi [HSR-TIGET, MILAN]

Neuronal and glial cells derived from a murine model of GM2-gangliosidosis display typical hallmarks of pathology and represent a valuable model to test gene therapy strategies

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M Hocquemiller [LYSOGENE, PARIS]

Widespread brain distribution of SGSH enzyme in canine brain following white matter injection of LYS-SAF302, an AAVrh.10-SGSH vector

REGENERATIVE THERAPIES

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B K Kaspar [NATIONWIDE CHILDREN'S HOSPITAL, COLUMBUS, OH]

Extensive dose ranging study of AVXS-101 in the severe d7 SMA mouse model

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C Bouquet [GENSIGHT BIOLOGICS, PARIS]

Humoral and cellular immune responses to AAV2 and ocular inflammation in patients after intravitreal injection of rAAV2/2-ND4 (GS010), an investigational gene therapy for the treatment of ND4 LHON

P212

D Sah [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

Selection of an AAV gene therapy targeting huntingtin for the treatment of Huntington's disease

REGENERATIVE THERAPIES

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C Bouquet [GENSIGHT BIOLOGICS, PARIS]

Toxicity, immunogenicity and biodistribution of AAV2.7m8-ChrimsonR-tdTomato (GS030-DP) gene therapy product after bilateral intravitreal administration in non-human primates

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M G Toscano [AMARNA THERAPEUTICS, LEIDEN]

SV40 gene delivery vectors provide effective transgene expression in the eye

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J Telfer [MEIRAGTX, LONDON]

Efficacy assessment and pre-clinical toxicology of AAV2/8-hCARp.hCNGB3, a CNGB3 gene therapy vector

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A Georgiadis [UNIVERSITY COLLEGE LONDON]

Pre-clinical toxicology of AAV2/5-OPTIRPE65, an optimised RPE65 gene therapy vector

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H Khabou [SORBONNE UNIVERSITÉS, UPMC UNIV PARIS 06, INSERM, CNRS]

Non-invasive foveal gene therapy for vision restoration : Making the blind see again

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J E Wagner [LUDWIG MAXIMILIAN UNIVERSITY MUNICH]

Preclinical evaluation of rAAV.CNGB1 in the Cngb1 knockout mouse model of Retinitis pigmentosa

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A J Smith [UNIVERSITY COLLEGE LONDON]

Efficacy and safety of AAV2/5-hRKp.RPGR to treat X-linked retinitis pigmentosa

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M Gasmi [ADVERUM BIOTECHNOLOGIES, INC., MENLO PARK, CA]

Long-term functional delivery of the human L-opsin cDNA via intravitreal administration of an AAV vector in Mongolian gerbils

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S Kim [YONSEI UNIVERSITY, SEOUL]

Inverted quasi-spherical three-dimensional cell culture systems for *ex vivo* gene-cell therapy

P232

J Ribeiro [UNIVERSITY COLLEGE LONDON]

Transplantation of mouse ESC-derived photoreceptor precursors into AIPL1-/- mouse model of retinal degeneration

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L Agundez [UNIVERSITY COLLEGE LONDON]

Exploring the potential of AAV-delivered CRISPR/Cas9 and iPS cells as a therapy for LCA-CEP290

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M Aristorena [UNIVERSITY COLLEGE LONDON]

AAV mediated gene therapy to treat loss of vision in CLN2 (Batten disease)

REGENERATIVE THERAPIES

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M Cobo [GENYO- UNIVERSITY OF GRANADA - JUNTA DE ANDALUCIA]

Preclinical studies of LentiVIP transduced mesenchymal stromal cells for the treatment of multiple sclerosis

P240

D Y Baek [YONSEI UNIVERSITY]

Combined application of induced neural stem cell, valproic acid and gene therapy in neurologic model

STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

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W L Di [UNIVERSITY COLLEGE LONDON, GREAT ORMOND STREET HOSPITAL]

Phase 1 feasibility and safety study of *ex vivo* lentiviral gene-modified epidermal grafts for Netherton syndrome

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R Blázquez [JUMISE, CÁCERES]

MSCs modulate the inflammatory response after surgical meshes implantation

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R Blázquez [JUMISE, CÁCERES]

TGF-ß mediated immunomodulatory activity of exosomes derived from endometrial mesenchymal stem cells

P252

P I Makarevich [LOMONOSOV MOSCOW STATE UNIVERSITY]

Adipose mesenchymal stromal cell sheets accelerate healing in rat model of deep wound

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G D Sagaradze [LOMONOSOV MOSCOW STATE UNIVERSITY]

Development of combined biomaterial based on human mesenchymal stem/stromal cell secreted products for spermatogenesis recovery

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O A Makarevich [LOMONOSOV MOSCOW STATE UNIVERSITY]

Evaluation of angiogenic properties of MSC conditioned media combined with collagen

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L Garcia Perez [LEIDEN UNIVERSITY MEDICAL CENTRE]

Reducing stem cell conditioning for SCID gene therapy

P260

A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Use of the active 3D matrix in combination with gene and cell therapy to stimulate skin regeneration

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F A Khafizova [KAZAN FEDERAL UNIVERSITY]

Application of nanostructural granules "nitigran" with mesenchymal stem cells in dentistry

P264

A Hochreiter [PARACELSUS MEDICAL UNIVERSITY SALZBURG]

Regenerative potential of periosteum-derived stromal cells

STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

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F A Khafizova [KAZAN FEDERAL UNIVERSITY]

The study of interaction between barrier membranes and soft tissues in the oral cavity of dogs

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H L Lee [YONSEI UNIVERSITY, SEOUL]

Hypoxia-specific VEGF-expressing system using erythropoietic enhancer lead to recovery of neuropathic pain

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K R Nash [UNIVERSITY OF SOUTH FLORIDA, TAMPA]

Expression of human UBE3a Via AAV5 recovers spatial learning and synaptic plasticity deficits in a model of Angelman syndrome

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A Follenzi [UNIVERSITY OF PIEMONTE ORIENTALE]

Application of combined gene and cell therapy within an implantable therapeutic device for the treatment of severe haemophilia A

P274

S Bobis-Wozowicz [JAGIELLONIAN UNIVERSITY, KRAKOW]

Hypoxia pre-conditioning enhances cardiac differentiation ability of human induced pluripotent stem cells

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E V Rozhina [KAZAN FEDERAL UNIVERSITY]

Enhanced dark-field microscopy for visualization of magnetic nanoparticles in multicellular spheroids

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STEM CELLS: BIOLOGY, MANIPULATION & REPROGRAMMING

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A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Cytochlasin B-induced membrane vesicles as vector for the bioactive molecules delivery

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L Mustapha [LAUSANNE UNIVERSITY HOSPITAL]

A concept of biocontainment for GMP cell production applied in a public hospital

P282

E V Rozhina [KAZAN FEDERAL UNIVERSITY]

Cell surface modification with polyelectrolyte and nanoclay

REGENERATIVE THERAPIES

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S Scala [HSR-TIGET, MILAN]

Clonal tracking of haematopoietic stem and progenitor cells *in vivo* in humans

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

Biologically active composition of human W8B2+ cardiac stem cell secretome

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O DelaRosa [TIGENIX SAU, MADRID]

Low risk of allo-sensitisation after intravenous administration of allogeneic adipose derived stem cells (ASCs)

P294

E Metzler [CHARITÉ MEDICAL UNIVERSITY AND THE MAX DELBRÜCK CENTRE FOR MOLECULAR MEDICINE, BERLIN]

iPSC generation and redifferentiation into satellite cells is dependent on cell type of origin?

REGENERATIVE THERAPIES

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S Szczypka Mark [PALL LIFE SCIENCES, PORT WASHINGTON, NEW YORK]

Cost of goods modelling for large scale mesenchymal stem cell expansion

P298

A B Carrillo-Gálvez [GENYO- UNIVERSITY OF GRANADA - JUNTA DE ANDALUCIA]

Glycoprotein A repetitions predominant (GARP) is a key molecule for the proliferation and survival of multipotent mesenchymal stromal cells (MSC): A potential role in the DNA damage response

P300

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

Placental MSCs and their derivatives as vehicles for the Na/I symporter (hNIS): A new antitumoral therapy

P302

V Capo [HSR-TIGET, MILAN]

Lentiviral vector gene therapy rescues bone resorption activity of TCIRG1-defective osteoclasts

P304

N Davoodian [HORMOZGAN UNIVERSITY OF MEDICAL SCIENCES, BANDAR ABBAS]

A protocol for production of functionally active hepatocyte-like cells from human adipose tissue derived stem cells

P306

M Pianella [GSK, BRENTFORD]

Evaluation of Asymptote's VIA Thaw for cryovials to achieve automated thawing of cryopreserved TF-1 cells

REGENERATIVE THERAPIES

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A A Rizvanov [KAZAN FEDERAL UNIVERSITY]

Modulation of the umbilical cord blood cells secretome using recombinant adenovirus encoding VEGF165

P310

I D Guryanov [KAZAN FEDERAL UNIVERSITY]

Polymer-stabilised magnetic nanoparticles as the instrument of cell transplantology

VECTOR DEVELOPMENT

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D Salas [UNIVERSITY OF NAVARRA, PAMPLONA]

Successful repeated hepatic gene delivery in non-human primates achieved with AAV5 by use of immune adsorption

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J Hartmann [PAUL EHRLICH INSTITUTE, LANGEN]

A screening procedure identifying DARPins suitable for the retargeting of lentiviral and AAV vectors

P320

N Meumann [HANNOVER MEDICAL SCHOOL]

Identification and characterisation of novel liver-directed AAV capsid variants exhibiting highly improved liver transduction features

P322

A P Dane [UNIVERSITY COLLEGE LONDON]

Analysis of the diversity of antibody response towards AAV8 capsid protein over time following a single administration of scAAV2/8-LP1-hFIXco in subjects with severe haemophilia B

VECTOR DEVELOPMENT

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E Horowitz [VOYAGER THERAPEUTICS, CAMBRIDGE, MA]

Enabling large-scale manufacturing of rAAV vectors with the baculovirus/Sf9 production system

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A Herrmann [HEIDELBERG UNIVERSITY]

Selection of AAV capsid libraries in cellular subpopulations within the liver

P328

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

AAV vectors conjugated to exosomes guide to more spreading and neuronal tropism; *in vivo* endomicroscopy assessment.

P330

K Benabdellah [GENYO, GRANADA]

The impact of SAR- and HS4-based insulators on the titer and transgene expression of non-integrating lentiviral vectors

P332

R Xicluna [INSERM U1089, NANTES]

Assessment of anti-AAV T cell responses against AAV 2, 4, 5, 8, and 9 serotypes in the general human population using the ELISpot-based technology

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Y Lad [OXFORD BIOMEDICA]

Large-scale production of lentiviral vectors for use in therapeutic applications

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M Penaud-Budloo [INSERM UMR 1089, NANTES]

The assembly-activating protein is essential for capsid assembly of AAV in insect cells

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VECTOR DEVELOPMENT

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Y Dickx [GENETHON, EVRY]
Identification of supplementary capsid-like proteins in various rAAV serotypes produced with the baculovirus/insect cell expression system

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