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# **AAV vector development**

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**Qiang Wang** University of Pennsylvania, Philadelphia, PA

Development of AAV3B variants with better liver transduction in nonhuman primates by directed evolution

#### P003

**Marti Cabanes-Creus** Children's Medical Research Institute

Restoring the intrinsic AAV2 human hepatotropism attenuated by tissue culture adaptation

## P005

**Cody Jackson** The Scripps Research Institute Development of a novel adeno-associated virus capsid for markedly enhanced muscle transduction

## P007

**Brian Kevany** Abeona Therapeutics

Development of a novel AAV capsid with improved PNS tropism for treating Pompe disease by intravenous administration

## P009

**Brian Kevany** *Abeona Therapeutics*Novel AAV capsids for delivery to the retina by intravitreal administration

#### P011

**Subha Karumuthil Melethil** *ReGenX Biosciences* 

Characterization of a novel AAV capsid with enhanced brain transduction following systemic delivery

# P013

James Warren Ultragenyx Gene Therapy Rapid CMC development and pre-commercial considerations for rAAV gene therapy products for rare diseases

## P015

**Erwan Sallard** *The University of Sydney* AAV p40 promoter expression in the absence of Rep proteins

## P017

**Silke Wissing** Cevec Pharmaceuticals GmbH, Cologne

Scalable AAV production using stable helper virus-free AAV producer cell lines based on CEVEC's CAP-GT cells

## P019

**Adrian Westhaus** Children's Medical Research Institute

Finding the perfect match: Directed AAV vector development guided by Targeted High Expression.

#### P021

**Caroline Odenwald** PROGEN Biotechnik GmbH

Cryo and negative staining EM – a comparison study with AAV capsids



**Veronique Blouin** *Université de Nantes* Advantages of homologous recombinationover transposition-based systems to generate recombinant baculovirus for AAV vector production

# P025

Marti Cabanes-Creus Children's Medical Research Institute

AAV development program: towards next generation of livertropic AAV variants

# P027

**Linas Padegimas** Abeona Therapeutics Development of an improved novel AAV capsids for intramuscular delivery

# P029

Joris van Arensbergen Gen-X B.V. SuRE: an unbiased, genome-wide assay to identify regulatory elements for custom-made gene expression

#### P031

**Peter Pechan** Solid Biosciences Evaluation of rAAV vectors engineered for muscle gene delivery

## P033

**Veronique Blouin** Université de Nantes Improving the high-throughput sequencing of adeno associated viral vector genome using an optimized PCR-free protocol

#### P035

**Susan D'Costa** Thermo Fisher Scientific Scalable chromatographic enrichment of full AAV capsids

# P037

Yohann Dickx Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE Molecular design optimization towards the development of a high scale rAAV8 production process using BEVS

## P039

**Elena lurlova** *Biotechnology company* BIOCAD, Saint-Petersburg

Development of new AAV extraction protocol for titration AAV using quantitative PCR and FLISA

# **Viral vectors manufacturing**

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Laura Garcia Perez Leiden University Medical Center

A new gene therapy method to assess transduction efficiency at the single-cell level.

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Cristina Nogueira Oxford BioMedica Development of secreted nucleases to eliminate residual DNA during viral vector manufacture

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## Cristina Peixoto iBET

Accelerating lentivirus manufacturing to the clinical

#### P047

Hanna Leinonen Kuopio Center for Gene and Cell Therapy Oy

Comparison of lentiviral vector production in two fixed-bed bioreactors

#### P049

**Josh Grieger** Asklepios Biopharmaceutial, Inc Development, optimization and scalability of the Pro10 rAAV manufacturing process

# P051

Giuliana Vallanti MolMed S.p.A. Lentiviral/Retroviral Vector large scale manufacturing

# P053

Adrien Soula Cell Therapy Catapult Development of a Scalable platform for AAV manufacturing

# P055

**Vincenzo Di Cerbo** *Cell Therapy Catapult* Single cell analysis of lentiviral integration to support ex-vivo gene modified cell therapy development

#### P057

**Corinne Branciaroli** Oxford Genetics Ltd Generation of cGMP-compliant stable packaging cell lines for inducible recombinant adeno-associated viral (rAAV) vector production

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Fulya Erendor Gene and Cell Therapy Center LentiVIP delivery suppresses systemic inflammation which results in beneficial outcome in Type 1 Diabetes

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**Keerang Park** Chungbuk Health & Science University

The establishment of highly sensitive real time-PCR-based detection methods of GMPmanufactured biological products for 14 human viral pathogens

#### P063

**Emanuele Bourges** Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE PTG1plus, a novel yransfection agent for a better AAV productivity in large-scale bioreactors

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# Ana Sofia Moreira iBET

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Mélissa Desrosiers Sorbonne Université Retrospective analysis of factors influencing AAV titers based on 1300 preparations.

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#### Ana Sofia Moreira iBFT

Integrating design of experiments to improve Lentiviral purification



Tom Payne Oxford Genetics Ltd Development of automated platforms for high-throughput screening of clonal viral packaging and producer cell lines

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Andrew Laskowski Pall Life Sciences Adherent HEK293t cells cultured in the Pall iCELLis® bioreactor with OptiPEAK HEK293t blood-free chemically defined media exhibit robust and rapid population doubling times

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**Keerang Park** Chungbuk Health & Science University

An in vivo method for detecting bovine herpes virus 1 as a possible adventitious contaminant utilising real-time PCR analysis

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Mégane Denu Polyplus Transfection High quality transfection reagents for therapeutic virus production

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**Jessica De Rooij** Thermo Fisher Scientific Accelerating advancement in gene therapy by improving downstream purification of viral vectors

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Maurizio Cattaneo Artemis Biosystems Continuous perfusion for an order of magnitude increase in lentiviral vector production

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Jessica de Rooij Thermo Fisher Scientific Addressing viral vector manufacturing challenges - innovative solutions in the AAV production workflow

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Luyuan Jin Beijing Stomatological Hospital, Capital Medical University

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Daniela Paasch Hannover Medical School Generation of Chimeric Antigen Receptor (CAR) macrophages from Human Haematopoietic Stem/Progenitor Cells (HSPCs)

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Eric Ehrke-Schulz Witten/Herdecke University,

Single adenoviral vectors armed with HPV oncogene specific CRISPR/Cas9 as efficient tumor gene therapy tools for HPV related cancers

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Marta Martinez-Lage Centro Nacional de Investigaciones Oncológicas CRISPR-mediated targeting of fusion oncogenes for cancer-directed therapy

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**Leysan Minigulova** Kazan Federal University Charge balance rule in the assembly of integral membrane proteins in tumor cells

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**Julia Davydova** *University of Minnesota* Clinical translation of a novel NIS-expressing oncolytic adenovirus for pancreatic cancer therapy and imaging

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Biagio De Angelis Ospedale pediatrico Bambino Gesù, Rome

A new promising CAR.CD30 T cell therapy associated with long persistence and high activity for treatment of CD30 positive lymphomas

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**Natasha Oppermans** *University of Manchester* Analysis of tumour reactivity and Vβ repertoire of melanoma TIL patients

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**Daria Chulpanova** Kazan Federal University Analysis of the interaction of mesenchymal stem cells overexpressing interferon alpha-17 and human neuroblastoma in co-culture in vitro

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**Jaitip Tipanee** Vrije Universiteit Brussel Validation of tumor suppressor microrna In liver tumorigenesis using hepatocyte-specific hyperactive piggybac transposons

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**Tomoko Ito** *Japan Anti-tuberculosis* Association, Shin-Yamanote Hospital Development of "Artificial neoepitope"presenting exosomes for novel cancer immunotherapy

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**Erica Carnevale** San Raffaele Scientific Institute, Milan

Generation of a library of WT1-specific T Cell receptors (TCR) for TCR gene edited T Cell therapy of acute leukemia

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## Rafael Moreno IDIBELL-ICO

Enhanced antitumor efficacy of armed oncolytic adenovirus-loaded menstrual blood-derived mesenchymal stem cells in combination with peripheral blood mononuclear cells

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Cellular viroimmunotherapy as a treatment to spontaneous canine supratentorial gliomas

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**John Rossi** *Kite, a Gilead Company* Pretreatment immunoscore and an inflamed tumour microenvironment are associated with efficacy in patients with refractory large B cell lymphoma treated with axicabtagene ciloleucel in ZUMA-1

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Samanta Romina Zanetti Josep Carreras Leukemia Research Institute, Barcelona CD19 and CD22-directed biespecific CAR for B-cell Acute Lymphoblastic Leukemia

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Yoshiyuki Koyama Japan Anti-tuberculosis Association Shin-Yamanote Hospital Antitumour immune activation by "artificial neoantigen"-presenting exosomes derived from the genetically modified cells

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Claudia Manuela Arnone Bambino Gesù Children's Hospital, Rome

Novel approach for treatment of pediatric high-grade gliomas through the combination of oncolytic adenoviruses and gene therapy encoding a BiTE directed to the EphA2 tumor antigen.

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**Chin Yang Chang** *Osaka University* HVJ-E in combination with CXCL2 suppressed murine melanoma through neutrophilmediated anti-tumor effect

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Franziska Köhler Institute for Anatomy, University Medicine Leipzig

Adeno-associated virus vectors (AAV) transduce human primary neurospheres and slice cultures of glioblastoma multiforme

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Alvaro Morales-Molina Instituto de Salud Carlos III

AKT and JUN are differentially activated in mesenchymal stem cells after infection with human and canine oncolytical adenoviruses

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**Shuji Kubo** Hyogo College of Medicine Human mesenchymal stem cells as tumorhoming cellular carriers of retroviral replicating vectors for cancer gene therapy

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Lara Herrera del Val Centro Vasco de Transfusión y Tejidos Humanos Adult peripheral blood and umbilical cord blood NK cells are good sources for effective CAR therapy against cd19 positive leukemic cells

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**Raul Torres** Centro Nacional de Investigaciones Oncológicas

CRISPR-mediated rearrangement generation of t(4;11) in human prenatal and perinatal haematopoietic stem/progenitor cells

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**Ilnur Ishmukhametov** Kazan Federal University

Intravital visualization of magnetic nanoparticles in eukaryotic cells using darkfield microscopy

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**Eleni Kalafati** University of Athens School of

The novel oncolytic lentiviral vector expressing IFNβ and pseudotyped with the measles virus HF glycoproteins displays therapeutic efficacy as a gene therapy-based approach for multiple myeloma

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Mikhail Durymanov Moscow Institute of *Physics and Technology* 

Influence of physiologically active agents on polyplex-mediated systemic gene delivery to melanoma tumours

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**Dirk Strunk** Paracelsus Medical University Salzburg

AML-derived extracellular vesicles transmit immunomodulatory potential

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**Kristina Havrysh** *Kazan Federal University* The combined action of cisplatin and physcion increases the ROS level in the lung and pancreas cancer cells

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**Bryan Strauss** Instituto do Cancer do Estado de Sao Paulo

Induction of cell death in human melanoma cell lines by the combination of p14ARF plus interferon-β gene transfer

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María Sancho Universidad de Zaragoza Cancer-derived exosomes loaded with ultrathin palladium nanosheets for targeted bioorthogonal catalysis

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Kristina Kitaeva Kazan Federal University Production of mouse mesenchymal stem cell lines with Luciferase and Katushka2s reporter gene expression for bioluminescence imaging

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**Kyuri Lee** Ewha Womans University Development of lipid nanoparticles for the mRNA-mediated cancer immunotherapy

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Kristina Havrysh Kazan Federal University SLC34A2 as a potential prognostic marker of triple-negative breast cancer patients' survival

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Kristina Kitaeva Kazan Federal University Analysis of Rac1, Bcl2, Cav1α, and Cav1β gene expression of neuroblastoma cells, mesenchymal stem cells and human peripheral blood mononuclear cells after co-cultivation



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**Gregory Cost** Casebia Therapeutics Expansion of genetically engineered regulatory T cells in vitro and in vivo via signaling through a synthetic, small-moleculecontrolled IL-2 receptor

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Allogenic CAR.CD19 natural killer cells: a new immuno-gene-therapy "off-the-shelf" in the treatment of acute lymphoblastic leukaemia.

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Daniel Abate-Daga H. Lee Moffitt Cancer Center and Research Institute yδ CAR-T cell therapy for bone metastatic castrate resistant prostate cancer

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Engineering NK cells to express nanobodybased Chimeric Antigen Receptors (CARs)

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Matteo Doglio San Raffaele Scientific Institute, Milan

CAR-Tregs for the treatment of Systemic Lupus **Ervthematous** 

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**Peer Heine** MaxCyte, Inc

Gene editing: Paving the way for accelerated clinical development of adoptive cell immunotherapies

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Roberta Sommaggio Veneto Institute of Oncology IOV – IRCCS, Padova

Antigen-specific targeting of triple negative breast cancer using Cytokine-Induced Killer cells redirected with monoclonal antibodies

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María Tristán-Manzano GENyO

LVs development for a fine-tuned regulation of CARs in T cells

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Development of an efficient transduction protocol for CAR-19 in cord blood derived-NK cells

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**Roland Leathers** Thermo Fisher Scientific PureQuant real-time PCR-based assay for quantitative determination of immune cell identity and purity

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**Arthur Bister** Heinrich Heine University Dusseldorf

Combining ROR1, CD5 or CD19 targeted chimeric antigen receptors reduces offtumour toxicity while maintaining lysis against double-positive mantle cell lymphoma cells

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Tatsuji Enoki Takara Bio Inc.

Development of robust T-cell expansion system and single-cell analysis of their expanded cells

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**Abeer ALhubaysh** University College London Investigating Optimal Storage Conditions in the Manufacture of CAR-T cells

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Aude-marie Alem JPT Peptide Technologies GmbH

A new transduction enhancer accelerates **CAR-T** production

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Eleni Papanikolaou Miltenyi Biotec, Bergisch-Gladbach

Automation in hemopoietic stem cell gene therapy: Results of a head-to-head comparison of a manual vs an automated procedure

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**Francesco Manfredi** San Raffaele Scientific Institute, Milan

Harnessing HLA-TCR interaction and the exhaustion signature for the isolation of tumor-specific T lymphocyte

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**Alessio Cantore** SR Tiget, Milan Liver-directed gene therapy with lentiviral vectors achieve normal levels of clotting factor VIII and IX in non-human primates

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Maria Carmina Castiello SR Tiget, Milan Non-genotoxic conditioning CD45-SAP promotes immunological reconstitution in a mouse model of Severe Combined Immunodeficiency caused by RAG1 defect

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Hai-jie Wang Shanghai Medical School of **Fudan University** 

Transplantation of lymphatic endothelial progenitor cells and sustained release of VEGF-C promote cardiac lymphangiogenesis after myocardial infarction

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Winston Vetharoy University College London Preclinical evaluation of the pCCLCHIM-p47 lentiviral vector for the gene therapy of p47phox deficient Chronic Granulomatous Disease



Mégane Brusson INSERM UMR 1163, Imagine Institute, Paris

A novel lentiviral vector for gene therapy of β-hemoglobinopathies: Co-expression of a potent anti-sickling transgene and a microRNA downregulating BCL11A

#### P213

**Verónica Palma Barqueros** *Universidad de* Murcia

Towards the lentiviral-mediated gene therapy for Glanzmann thrombasthenia

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**Bridget Yates** BioMarin Pharmaceutical Vector genome processing of AAV5-hFVIII-SQ in mouse and monkey livers

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Carlos Carrascoso-Rubio CSIC/UAM, Madrid Preclinical studies of a gene therapy approach for the bone marrow failure in X-linked dyskeratosis congenita

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Antonella Bastone Hannover Medical School Development of a new all-in-one in vitro safety assay for gene therapy

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Sami Jalil University of Helsinki Increasing fetal hemoglobin by genetic editing of sickle cell disease patient haematopoietic stem cells

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**Baolei Yuan** King Abdullah University of Science and Technology

Complete knockout of the Wiskott-Aldrich syndrome gene impairs phagocytosis and chemotaxis and enhances proliferation in macrophages

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Mathieu Streefland Kiadis Pharma. Amsterdam

Development of a fully-closed and automated manufacturing process for ATIR101: Donor lymphocytes depleted of host alloreactive T-cells

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Flaminia Clelia Lorenzetti Medical Genetics. University of Siena

Patient-specific TP53 mutations CRISPR-Cpf1 editing in chronic lymphocytic leukemia by suicide gene delivery

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Maria Jose Sanchez CABD, CSIC, UPO, Seville Fetal liver haemato/vascular progenitor cells as a cell-based therapeutic tool for neonatal haemophilia

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**Roland Leathers** Thermo Fisher Scientific StemPro<sup>™</sup> HSC expansion medium supports superior expansion of human haematopoietic stem-progenitor cells

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Michaela Öller Paracelsus Medical University Salzburg

Human plasma and platelets from neonatal and adult blood express differential sets of regenerative proteins

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Michaël Hocquemiller Lysogene LYS-SAF302 gene therapy study in mucopolysaccharidosis type IIIA (mps IIIA) children

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**Freja Ekman** *University of California, Berkeley* CRISPR-Cas9-mediated genome editing improves motor deficits and lifespan in a mouse model of Huntington's disease

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Yoshie Kurokawa Jichi Medical University Gene therapy in a murine model of Niemann-Pick disease type C

## P247

**Lodewijk Toonen** *uniQure biopharma B.V.* A novel AAV-based miQURE gene therapy for SCA<sub>3</sub>

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**Assumpcio Bosch** *Univeristat Autònoma de* Barcelona

Broad and specific expression of Glial-Derived Neurotrophic Factor (GDNF) in muscles as gene therapy strategy for Amyotrophic Lateral Sclerosis

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**Sara Marcó Costa** *Center for Animal Biotechnology and Gene Therapy* Safe and sustained elevation of sulfamidase after administration of AAV9-sulfamidase to the CSF of dogs: Seven year-follow up

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**David Scott** Aspa Therapeutics

A route of administration study of BBP-812, an AAV9-based gene therapy for the treatment of Canavan disease, in juvenile cynomolgus macaques

## **P255**

Françoise Piquet INSERM U1127, *NeuroGenCell, Brain and spine Institute, Paris* Restoring neuronal cholesterol efficiently rescues ALS mouse model

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**Kamal Gadalla** *University of Edinburgh* Gene transfer rescues established phenotypes in a mouse model of Rett syndrome

## **P259**

Annita Montepeloso Harvard Medical School, Boston, MA

Improving the ability of haematopoietic stem cells to generate a microglia-like progeny upon transplantation



# Matthew Meriggioli AveXis, Inc.

Adeno-associated virus serotype 9 (AAV9) antibodies in patients with spinal muscular atrophy (SMA) screened for treatment with onasemnogene abeparvovec

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Lucía Mora Jiménez CIMA, Universidad de Navarra

Delivery of the SCN1A into the brain using High-Capacity Adenoviral vectors for the treatment of Dravet Syndrome

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Marisa Cappella Sorbonne Université, Inserm UMRS 974, Institut de Myologie

AAV-mediated expression of antisense oligonucleotides for the treatment of C9orf72-ALS

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# Michaël Hocquemiller Lysogene

PROVide: Video based Patient Reported Outcomes for Sanfilippo Syndrome (MPS IIIA): A new and innovative approach to record and measure disease post gene therapy.

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# Kelly Glajch Biogen

AAV gene therapy for Parkinson's disease: in vitro and in vivo effects of AAV-GBA treatment in GBA mutant models

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Valentina Poletti Harvard Medical School.

Long term effect of prostaglandin E2 exposure on haematopoietic stem/progenitor cells

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**Eva Hede Olsen** Aalborg University

Gene therapy to the blood-brain barrier with resulting protein secretion as a strategy for treatment of NPC2 disease

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**Joan Roig Soriano** *Univeristat Autònoma de* Barcelona

AAV-mediated over-expression of secreted and transmembrane aKlotho isoforms reverse cognitive and molecular aging hallmarks in senescent mice

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# Francesca Ornaghi SR Tiget, Milan

Optimized bicistronic lentiviral vectors to correct β-hexosaminidase deficiency in neural and haematopoietic stem/progenitor cells and progeny

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Beihui Liu Oxford BioMedica

Targeting neurodegeneration using lentiviral gene therapy

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**Natalia Bal** Institute of Higher Nervous Activity and Neurophysiology of RAS, Moscow DNA cytosine methylation contributes to long-term memory maintenance in snails

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**Ilnur Ishmukhametov** *Kazan Federal* University

Pharmacological protection of the spinal cord in the first hours after injury

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Investigation of microglia cells behavior in spinal cord injury of varying severity

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**Shubham Maurya** *Indian Institute of* Technology Kanpur India

Ocular gene therapy with a synthetically engineered AAV enhances visual function in a pre-clinical model of Leber congenital amarousis

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## **Bernard Schneider**

École Polytechnique Fédérale de Lausanne Delivery of CRISPR/Cas9 using AAV-PHP.B in the inner ear leads to allele-specific inactivation of the mutated Tmc1 allele and protects auditory function in Beethoven mice

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William Beltran University of Pennsylvania, Philadelphia, PA, USA

Long-term efficacy of rhodopsin knockdown and replacement gene therapy in a canine model of autosomal dominant retinitis pigmentosa

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**Rob Collin** Radboud University MC Splice modulation therapy for a variety of ABCA4 mutations underlying Stargardt disease

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# Fabio Dell'Aquila TIGEM

Towards a clinical trial of gene therapy for Usher syndrome type IB retinitis pigmentosa

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**Carrie Margulies** *Editas Medicine* 

Developing a CRISPR/Cas9 editing approach for the treatment of USH2A-related inherited retinal degeneration

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Gaelle Chauveau Université d'evry Subretinal injection of rAAV2/8 vector induces dose-dependent retinal alteration and systemic anti-transgene T-cell response similarly in wild type and pathophysiological murine models

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Ning Chang Jules-Gonin Eye Hospital, UNIL Towards the gene therapy for FAM161A associated retinitis pigmentosa in a murine model

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ABCA4 gene dysfunction in Stargardt retinal disease: Charcaterisation of two new rat transgenic models



# **Martina Kropp** HUG

Ex vivo efficacy study to evaluate neuroprotection in a retinal organotypic culture system: A gene therapeutic strategy to deliver growth factors to the retina to treat avascular age-related macular degeneration

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Nina Harmening Université de Genève Establishment of an in vivo protocol for nonviral gene delivery to ocular tissue

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Tae Kwann Park Soonchunhyang University Hospital Bucheon

A novel modified deep intravitreal injection improves Adeno-associated virus (AAV)mediated retinal transduction

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# Birgitte Volck AVROBIO, Inc

AVR-RD-01 lentiviral gene therapy reduces Gb3 substrate in endothelial cells of renal peritubular capillaries, in a previously untreated classic Fabry male patient

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Transgene immunity following AAV-mediated gene transfer to the liver is associated to persisting viral genomes and T cell exhaustion

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**Irene Ros Gañán** *Vivet Therapeutics* Sensitivity of different AAV serotypes to preexisting NAbs

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## Mark Shearman AGTC

Neutralizing anti-AAV antibody impact on vector transduction following intravitreal administration of AAV in non-human primates

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## Kate Lewis bluebird bio

A retrospective literature review of the safety outcomes of clinical studies of haemotopoietic stem cell gene therapy using lentiviral vectors in non-oncologic diseases

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**Gregory Hayes** BioMarin Pharmaceutical Treatment with valoctocogene roxaparvovec in a Ph1/2 study (BMN 270-201) elicits crossreactive antibodies against divergent AAV capsids that exhibit different kinetic profile than anti-drug antibodies.

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**Sungho Shin** *Seoul National University* Induction of humoral and cellular immune responses in mice immunized with a DNA vaccine for Zika virus

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Elaina Breznau Charles River Laboratories Robust solutions for navigating challenges associated with development and validation of cell-based neutralising antibody assays

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**Geoffrey Casey** University of Alberta Innate immune response of the retinal pigment epithelium to gene therapy vectors

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**Linas Padegimas** Abeona Therapeutics Novel AAV capsids show increased evasion to wild-type AAV9 neutralizing antibodies

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R Watano Jichi Medical University Prevalence of NAb against AAV8 in micromini pigs

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**Sergei Abramov** *Kazan Federal University* Detection of macro-throtropin in patients with Hashimotos Thyroiditis and subclinical hypothyroidism

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Marco Zahn GeneWerk GmbH, Heidelberg Evaluation of T- and B-cell receptor diversity using different immune repertoire sequencing methods

# iPSC & organoids, research and therapy developments

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Scalable allogeneic cell therapies derived by reprogramming inducibly-immortalised adult stem cells

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**Andres Miguez** University of Barcelona In vivo progressive degeneration of Huntington's disease patient iPSC-derived neurons reveals human-specific pathological phenotypes

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## **Jean-Baptiste Dupont** *I-STEM*

Single cell developmental trajectories reveal early phenotypes in Duchenne muscular dystrophy prior to skeletal muscle commitment

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María Martín López Andalusian Network for Design and Translation of Advanced Therapies Modelling chronic cervical spinal cord injury in aged rats for cell therapy studies



## Giulia Raimondi IDIBAPS

Patient-derived pancreatic tumor organoids identify therapeutic response to oncolytic adenovirus

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Innovative 3D model for the establishment of primary paediatric low-grade glioma cultures: New platform for the preclinical study of immunotherapeutic approaches

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# Silvia Pascual IDIBAPS

Establishment of a 3D co-culture model for the study of tumour-stroma interactions in pancreatic ductal adenocarcinoma

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Belén Alvarez-Palomo Banc de Sana i Teixits IPS-PANIA project to develop a HLA matched bank of iPSC in Spain

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**Denise Klatt** Hannover Medical School Targeting the AAVS1 locus for genetic correction of p47-CGD in iPSC revealed differential transgene silencing of myeloidspecific promoters

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**Paul Gissen** UCL Institute of Child Health Bioreactor grown iPSc derived hepatocyte-like cells for testing liver directed gene therapy

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Generation of genetically modified osteoclasts from induced pluripotent stem cells derived from an infantile malignant osteopetrosis patient with a TCIRG1 mutation

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Josse Depla uniQure biopharma B.V. Stem cell derived brain organoids, a promising model to study Adeno associated viruses for CNS gene therapy

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**Louise Bullen** *Manchester Metropolitan* University

Interrogation of the molecular role of p62 in iPS reprogramming and maintenance of pluripotency

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Pall Xpansion® Bioreactor supports progenitor cell growth to >1 million cells/cm2 and proper cell differentiation

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Generation of human microglia-like cells from PD patient-specific iPS cells

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Maria Usik SSC RF Institute for Biomedical Problems RAS

The acetylase and deacetylase content in mice ovaries, testes, heart and lung tissues under modeling weightlessness

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**Akaitz Dorronsoro González** Instituto de Investigación Sanitaria La Fe

Extracellular vesicles with enhanced immunosuppression capacity secreted by genetically improved mesenchymal stem cells derived from dental pulp

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**Yoojin Seo** Pusan National University Enteroendocrine cell-derived hormone A is involved in the intestinal homeostasis by directing the differentiation of the mouse intestinal organoid

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**Kirill Sukhinich** Russian Academy of Sciences, Moscow

Regenerative effects of solid neural tissue grafts located in gelatin hydrogel conduit for treatment of peripheral nerve injury

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Exploiting targeted epigenetic editing to increase the yield, homogeneity and purity of human iPSC- derived oligodendrocyte cell populations

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Marina Rodríguez Rubio Severo Ochoa Molecular Biology Research Center Study of the effect of electrical stimulation in brain development using human brain organoids

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Reconstruction of neuronal network by transplantation of human skin-derived iPS cells after brain stroke

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Pulmonary macrophage transplantationbased therapy for alpha-1 antitrypsin deficiency

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Mauro Giacca ICGEB. Trieste

Novel cardioactive factors selected in vivo from an AAV library encoding the secret

# P409

**Eleonora Leggiero** CEINGE-Biotecnologie Avanzate, Napoli

Muscle expression of a secreted LDLR/ Tfchimeric proteinameliorates lipid profile inLDLR-deficient mice



Anastasia Efimenko Lomonosov Moscow State University

Decellularized extracellular matrix in modelling lung fibrosis development in vitro

# Liver gene and cell therapy

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Reynald Lescarbeau Intellia Therapeutics In vivo delivery of CRISPR/Cas9 to the liver using lipid nanoparticles enables gene knockout in multiple species

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**Jooyoung Lee** *University of Ulsan College of* Medicine

Differentiation of human liver-derived stem cells into hepatic lineage for therapeutic liver reconstitution

## P419

Michela Milani SR Tiget, Milan Targeting of hepatocyte subpopulation contributing to liver post-natal growth is crucial for maintenance of transgene expression in liver-directed gene therapy

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Richard Torres BioMarin Pharmaceutical Structural characterization of AAV5-FVIII-SO vector DNA in human blood by Real-Time and Droplet Digital™ PCR

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Qiang (John) Xiong LogicBio Therapeutics Durability of factor IX expression in mice treated neonatally with a nuclease-free, promoterless, AAV-based gene therapy, GeneRide™

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**Pei She Loh** National University of Singapore, Singapore

Design, investigation and delivery of advanced trans-splicing RNA for suicide gene therapy

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Marta Parés Casellas Vall d'Hebron Research Institute

Preclinical assessment of a hepatocytedirected gene editing approach based on viral vectors and polymeric nanoparticles

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Daisuke Kami Kyoto Prefectural University of Medicine

Cell therapy for Fabry disease using CellSaic technology

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Laia Tolosa Health Research Institute La Fe (IIS La Fe)

Human hepatocytes encapsulated in injectable hydrogels for their use in liver cell therapy

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**Shinnosuke Tsuji** *Stanford University* Elucidating the mechanism of species specificity of recombinant AAV-LK03 transduction

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Lorelei Stoica Ultragenyx Pharmaceutical Using AAV vectors to deliver ATP7B as a treatment for Wilson disease

# **Metabolic diseases**

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**Marshall Huston** Sangamo Therapeutics Liver-targeted AAV gene therapy vectors produced at clinical scale result in high, durable levels of α-GalA enzyme activity and effective substrate reduction in a mouse model of Fabry disease

## P443

**Sean Armour** *Spark Therapeutics* Pre-clinical development of SPK-3006, an investigational liver-directed AAV gene therapy for the treatment of Pompe disease

## P445

**Jamie Benoit** Axovant Sciences AXO-AAV-GM1 for the treatment of GM1 gangliosidosis: Program overview

#### P447

**Natalia Gomez-Ospina** *Stanford University* Human genome-edited haematopoietic stem cells phenotypically correct Mucopolysaccharidosis type I

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Noelia Benetó University of Barcelona, CIBERER, IBUB, IRSJD, Barcelona Novel neuronal and astrocytic models for Sanfilippo C syndrome using CRISPR/ Cas9- edited iPSC: Their use in therapeutic approaches.

## P451

Giulia De Sabbata ICGEB. Trieste A novel AAV-based therapy in combination with tolerogenic ImmTOR nanoparticles for a sustained treatment of ornithine transcarbamylase deficiency

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Lina Li NHGRI, NIH, Bethesda, MD The combination of ImmTOR with AAV Anc80 is therapeutically effective, safe, and repeatable in mice with methylmalonic acidemia while also being compatible with the low seroprevalence of Anc80 Nabs in the patient population

# P455

**Claudia Jambrina Pallarés** *Center for Animal Biotechnology and Gene Therapy* Skeletal muscle-directed FGF21 gene therapy counteracts obesity and type 2 diabetes

# P457

**Gloria Gonzalez-Aseguinolaza** Center for Applied Medical Research

Preclinical validation of radioactive copper excretion as a translational tool for evaluating the pharmacodynamics of gene therapy VTX-801 for Wilson's disease in future first in human



**Javier Torres Torronteras** Vall d'Hebron Research Institute, Universitat Autònoma de Barcelona

The alpha-1-antitrypsin promoter improves the efficacy of an AAV vector for the treatment of MNGIE

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**Bridget Yates** BioMarin Pharmaceutical Phenylalanine hydroxylase (PAH) liver distribution and characterization following AAV5-hPAH gene therapy in Pahenu2 mice

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Salih Sanlioglu Akdeniz University Center for Gene and Cell Therapy

Lentivirus mediated insulin promoter directed insulin gene expression is effective in suppressing postprandial glucose excursions in Type 1 diabetes

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Rocio Maldonado University of Helsinki Gene edited cells in Mitochondrial Recessive Ataxia Syndrome (MIRAS)

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**Gizem Seker** Akdeniz University Center for Gene and Cell Therapy

Lentiviral-mediated TRAIL transfer decreased serum alkaline phosphatase, total cholesterol, and glucose levels in high-fat diet-fed obese mice

#### P469

**Zhaochen Shan** School of Stomatology, Capital Medical University

S1P prevent irradiation induced parotid injury in miniature pig model

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Ahter Dilsad Sanlioglu Akdeniz University Center for Gene and Cell Therapy TRAIL increases glucose-stimulated insulin

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secretion in Min6 cells

**Jennifer Ana Pérez Castro** Center of Animal Biotechnology and Gene Therapy

Long-term correction of visual impairment after AAV-NAGLU-mediated gene therapy in a mouse model of mucopolysaccharidosis type IIIB

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## P483

Francesco Saverio Tedesco University College London

Modulating cell fate determinants for muscle cell therapies: Notch and PDGF signaling enhance stemness and migration of satellite cells and human iPS cell-derived myogenic progenitors

#### P485

Ignacio Perez de Castro Instituto de Salud Carlos III

Study on the therapeutic potential of HITI for the treatment of LMNA-associated congenital muscular dystrophies

#### P487

Camille Vaubourg Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE Effect of Smad7 gene transfer in a Duchenne muscular dystrophy mouse model

# P489

**Shelby Hamm** *Virginia Tech* Effects of exercise on the efficacy of microdystrophin gene therapy

#### P491

**Oleg Gusev** *Kazan Federal University* Expression of L-type Ca+2 channels in m. Soleus and m. EDL of rats at early stages of hind-limb unloading and upon their subsequent re-adaptation

## P493

Nilakshi Dhananjani Dhanushika **Ratnayake** Australian Regenerative Medicine Institute

Macrophage secreted factors as novel therapeutics to promote stem cell-mediated muscle repair

#### P495

Penléope Romero Duque Vall d'Hebrón Institut de Recerca (VHIR), Barcelona Development of a novel preclinical gene therapy approach for congenital muscular dystrophy 1A

## P497

Alina Cequier Soler University of Zaragoza Humoral immune response against allogeneic equine mesenchymal stem cells (MSCs) mediated by the major histocompatibility complex (MHC): An issue to take into account for the safety and efficacy of treatment with **MSCs** 

# P499

Ilnur Ishmukhametov Kazan Federal University

Differentiation potential of stem cells cultured on glass surfaces coated with magnetic nanoparticles

#### P501

Viktorija Cernisova Royal Holloway, University of London

Periostin splice variant expression in skeletal muscle fibrosis and effective anti-fibrotic action of antisense oligonucleotides that block periostin expression

#### P503

Ivan Yakovlev Human Stem Cells Institute. Moscow

Assessment of exon-skipping feasibility for treatment of dysferlinopathy

# P505

Nastasia Kosheleva FSBSI Institute of General Pathology and Pathophysiology, Moscow Mesenchymal stem cells from gingiva as a new source of autologous myogenic progenitors



Yong-Gun Kim Kyungpook National University Visualization of woven bone structure through analysis of biopsy specimens using synchrotron radiation and conventional X-ray micro-CT

# P509

**Olga Chernova** Kazan Federal University Histopathological analysis of gingiva-derived MMSCs transplantation effect on skeletal muscles regeneration

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**Ying Zheng** Capital Medical University Evaluation of collagen membrane as a pulp capping agent in swine

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**Ilnur Ishmukhametov** Kazan Federal University

Chondrogenesis of stem cells on substrate coated with magnetic nanoparticles

## P515

Maria Boldyreva Russian Ministry of Health, Moscow

Adipose stromal cell sheet producing hepatocyte growth factor (HGF) effectively stimulates recovery of ischemic skeletal muscle in mouse hind limb ischemia model

#### P517

**Lijia Guo** Capital Medical University microRNA 21 promotes orthodontic tooth movement via TNF-alpha/RANKL pathway in T cells

#### P519

**Artur Fedianin** Kazan Federal University The rat skeletal muscle under the simulated weightlessness

# P525

Anastasia Efimenko Lomonosov Moscow State University

Diversity of mesenchymal stromal cells: Secretome in focus

# Skin gene and cell therapy

## P531

Ai-Li Shiau National Cheng Kung University Medical College

Knockdown of prothymosin α improves wound healing in diabetic mice

#### P533

**Rodolfo Murillas Angoiti** Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz IIS-FJD

Preclinical models for in vivo gene editing of COL7A1 based on delivery of CRISPR/Cas9 to RDEB patient skin by adenoviral vectors

## P535

Araksya Izmiryan INSERM UMR 1163, Imagine Institute, Paris

CRISPR/Cas9-mediated correction of two recurrent COL7A1 mutations in primary and induced pluripotent stem cells from patients with recessive dystrophic epidermolysis bullosa

#### P537

Nataliya Basalova Institute foLomonosov Moscow State University Extracellular vesicles secreted by

mesenchymal stromal/stem cells reverse TGF-β induced myofibroblast differentiation

# P539

Anastasia Efimenko Lomonosov Moscow State University

Antifibrotic effect of mesenchymal stromal cell sheets is mediated by interaction of stromal and endothelium cells

## P541

# **Christine Baldeschi** *I-STEM*

Production of clinical grade temporary epidermal substitute obtained from hESC derived keratinocytes for the treatment of sickle cell leg ulcers: a challenge for regenerative medicine

#### P543

Leire Gardeazabal I.I.S. Biodonostia Human breast milk exosomes accelerate mouse wound healing

# New approaches in gene editing

# P549

**Asael Herman** *Emendo Biotherapeutics* Target optimized variant of CRISPR associated nuclease enables allele-specific knock out of ELANE-related neutropenia

#### P551

Sara Fañanás Baquero CIEMAT / CIBERER,

Efficient gene editing of the PKLR locus in human long-term haematopoietic stem cells to correct Pyruvate Kinase Deficiency

# P553

**Andreas Neerincx** Bayer AG Preclinical Research, Protein Engineering, Cologne Engineering of small and potent synthetic RNA-quided cytosine base editors

## P555

Oscar Quintana Bustamante CIEMAT / CIBERER, Madrid

Highly precise gene editing correction of a Pyruvate Kinase deficiency-causing mutation in patient-derived lymphoblastic cells using single stranded oligodeoxinucleotides

## P557

**Yongxing Fang** *University of Freiburg* Chimerization enables effective delivery of TALE-based effectors by lentiviral vectors

#### P559

**Beatrice Claudia Cianciotti** San Raffaele Scientific Institute, Milan

LAG-3, but not Tim-3, disruption in TCR gene edited human memory stem T cells enhance the anti tumor activity against multiple myeloma



Simone Haas University of Freiburg Abnoba-seq: A sensitive in vitro assay to profile CRISPR-Cas nuclease off-target activity in a truly unbiased manner

# P563

## Mollie Schubert IDT

Improved methods for CRISPR HDR using Alt-R HDR Enhancer and modified ssDNA donors with optimized design

## P565

**Aurelien Jacob** SR Tiget, Milan Cell cycle "push-up" to boost targeted genome editing in haematopoietic stem cells

#### P567

Suki Roy Nanyang Technological University, Singapore

A novel λ integrase-mediated transgenesis platform for biologics production

## P569

**Florine Samain** *Flash Therapeutics* All-in-one delivery using LentiFlash technology, a MS2-chimeric RNA delivery tool designed for clinical applications

#### P571

**Stefania Crippa** *SR Tiget, Milan* Mesenchymal stromal cell support to optimize HSPC-gene editing

#### P573

Roberto Nitsch AstraZeneca. Biopharmaceutical R&D

Assessment of the tumorigenicity potential of CRISPR/Cas9 genome editing using an innovative in vitro cellular transformation assay

#### P575

Natalia Gomez-Ospina Stanford University Engineering monocyte lineage-specific glucocerebrosidase expression in human haematopoietic stem cells using genome editing: A universal strategy for genetic correction in Gaucher disease

#### P577

**Duran Sürün** Dresden University Base editors-mediated generation of point mutations in IPS cells

## P579

Araceli Aguilar González GENyO Generation of cellular models to study gene therapy strategies for Pompe disease

#### P581

Marta Zinicola Institute of Child Health Targeted gene insertion for the treatment of X-linked agammaglobulinemia (XLA)

## P583

**Amy Walker** *University College London* Nanoparticle delivery of CRISPR/Cas9 for treatment of cystic fibrosis by homology independent targeted integration

#### P587

Virginia Arechavala Biocruces Bizkaia Health Research Institute

A CRISPR/Cas9 edition protocol for human myoblasts to generate disease models

# P589

**Lucrezia Della Volpe** Universita Vita-Salute San Raffaele

Cell cycle dependent activation of a proinflammatory transcriptional program in Haematopoietic Stem and Progenitor cells (HSPCs) in response to DNA damage

## P591

**Dawid Glow** *University Medical Center* Hamburg-Eppendorf

Novel cell-based assays for the assessment of potential toxic effects of CRISPR/Cas9 genome editing

## P593

Maria Schacker Biopharma Excellence From fiction to science: Clinical potentials of gene editing

#### P595

**Gaurav Agrahari** Catholic University of Korea Superoxide dismutase 3 augments survival rate of mesenchymal stem cells in the nutritionally stressed condition

#### P601

**Deya Corzo** Sigilon Therapeutics Correcting bleeding disorders using blood clotting factors produced in vivo by shielded engineered allogeneic cells

#### P603

Youna Coquin Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE Lentiviral vectors pseudotyped with murine syncytins efficiently transduce B cells in vitro and in vivo

# **New delivery systems**

## P605

**Branden Moriarity** *University of Minnesota* Engineered B cells as a universal platform for the treatment of enzymopathies

## **P607**

**Emilie Audouard** INSERM U1127 -**NEUROGENCELL** - Eq Cartier

Therapeutic approach for multiple sclerosis using a bioelectronic cell implant

## P609

**Anne Galy** *Genethon, UMR\_S951, Inserm, Univ* Evry, Université Paris Saclay, EPHE Human endogenous retroviral envelope glycoproteins syncytin-1 and syncytin-2 enable effective lentiviral transduction of human primary B cells and dendritic cells

# P611

Daniel Veilleux EnGene Inc.

Development and pre-clinical characterization of an oral solid dosage formulation for gene delivery to intestinal mucosa



Cristina Fornaguera Institut Quimic de Sarria Poly(beta aminoester) nanoparticles encapsulating mTOR siRNA for lung cancer therapy

# P615

**Christopher Thornton** *Manchester* Metropolitan University Safer and more stable iPSC generated using Doggybone DNA vectors

## **P617**

**Robin Stiener** Ulm University Enabling efficient transduction of mesenchymal stromal cells by HAdV-5-based vectors

## P619

Yumiko Komatsu Kyoto University *In vivo* evaluation of replication competent and defective RNA-virus based episomal vector system

#### P021

Alicia Rodríguez Gascón University of the Basque Country UPV/EHU

Effect on alpha-Gal A activity of shortterm systemic therapy with a solid lipid nanoparticle based non-viral vector in a knockout mouse of Fabry disease

## P623

Heleen van der Veen Utrecht University Microfluidic ynthesis of ionizable lipid nanoparticles containing CRISPR/Cas9 ribonucleoprotein complexes

## P625

**Sofia Shtykalova** Laboratory of Molecular Genetics and Gene Therapy, Saint-Petersburg Suicidal gene therapy of leiomyoma via delivery of herpes thymidine kinase gene by means of αvβ3 integrin-targeted peptidebased nanoparticles

## P627

**Albert Rizvanov** *Kazan Federal University* Recombinant plasmids containing picornaviral self-cleaving 2A-peptides and expressing VEGF and FGF2 growth factors induce angiogenesis in vivo

#### P629

**Ezgi Oner** *Ege University, Izmir* Evaluation of the cationic solid lipid nanoparticles carrying siRNA against EphA2 receptor as non-viral delivery systems

## P631

Franziska Hausig Friedrich Schiller University Jena

Novel non-toxic polymers for overcoming the endosomal barrier

# P633

**Shauna Dauphinee** *EnGene Inc* 

Optimization of a nanoparticle gene delivery system for intravesicular delivery of DNA to bladder mucosa

## P635

Matt Krusen Aldevron

mRNA synthesis reagents and manufacturing: Research through clinical development

#### P637

Gilta Jaeckel Charles River Laboratories Validation of a relative standard curve RT-qPCR method for the measurement of a therapeutic microRNA in plasma and tissue samples

# P639

**Busra Cesur** Akdeniz University Center for Gene and Cell Therapy

Development of nano-sized non-viral DNA delivery system for gene therapy purposes

## P641

Ilnur Ishmukhametov Kazan Federal University

Magnetically modified halloysite nanotubes nanocontainers for drugs

# P643

Ilnur Ishmukhametov Kazan Federal University

Selective DNA-damaging activity of curcumin@halloysite nanoformulation

#### P645

Maxim Baltin Kazan Federal University Effect on the electrical characteristics of spontaneously active neurons of the visceral ganglion of the nervous system of the mollusk Helix lucorum

## P647

**Diana Sabirova** Kazan Federal University Evaluation of methylprednisolone delivery to nerve tissue using polymer conjugates in spinal cord injury in rats

#### P649

Ilnur Ishmukhametov Kazan Federal

Relationship between oil-degrading bacteria Alcanivorax borkumensis and nematode Turbatrix aceti

# **Regulatory session**

# P655

**Daniel O'Connell** Intellia Therapeutics In silico, biochemical and cell-based integrative genomics identifies precise CRISPR/Cas9 targets for human therapeutics

#### P657

**Shih-Yao Chen** *National Cheng Kung* University Medical College Galectin-3 as a NETosis mediator in systemic lupus erythematosus

## P659

**Sheridan Sarah** *Merck Millipore* Biosafety testing of cell and gene therapies; rapid methods and regulatory expectations

#### P661

**Carolina Iglesias-Lopez** *Universitat* Autònoma de Barcelona

Hurdles of environmental risk assessment procedures for advanced therapies medicinal products

#### P663

Florian Durst Thermo Fisher Scientific Streamlined high performance extraction and quantitation of host cell residual DNA



Joel Montané Mogas Asphalion Regulatory and nonclinical considerations for translation of gene therapy medicinal products to first-in-human clinical trials

# P667

Elisabet Aguilar Andalusian Network for Design and Translation of Advanced Therapies Scalability strategies for cell manufacturing

#### P669

**Katie Snell** UCL Institute of Child Health Developing a standard operating procedure for clinical trial advanced therapy investigational medicinal product administration; a single centre experience

# Other

## P671

María Rosario Hervás Salcedo CIEMAT / CIBERER, Madrid Enhanced anti-inflammatory properties

of human mesenchymal stromal cells by transient co-expression of CXCR4 and IL-10

# P673

Yen-Hui Chan Taichung Tzu Chi Hospital Transcriptomic characterization and gene therapy of balancing dysfunction in Slc26a4defected mice

#### P675

**Esther López** Centro de Cirugía de Mínima Invasión Jesús Usón

Bone marrow mesenchymal stem cells accelerate wound healing after hernia repair with polypropylene surgical meshes

# P677

**Natalia Izotova** *UCL Institute of Child Health* De novo production of genetically engineered T cells is maintained in humans decades after loss of transplanted haematopoietic stem cells.

## P679

Luca Del Core SR Tiget, Milan

A novel Bayesian regression framework highlights dependences of clonal dynamics on different vector designs in in vivo gene therapy studies

#### P681

Fabrizio Benedicenti SR Tiget, Milan Sonication Linker Mediated-PCR (SLiM-PCR), an efficient method for quantitative retrieval of vector integration sites

## P683

Mercedes Lopez-Santalla CIEMAT / CIBERER, Madrid

Cell therapy with adipose-derived mesenchymal stromal cells alters the endogenous trafficking of myeloid populations in vivo

#### P685

**Qingsong Jiang** Beijing Stomatological Hospital, Capital Medical University Depletion of PTN inhibits the osteogenic differentiation potential of dental pulp stem cells

# P687

**Esther López** Centro de Cirugía de Mínima Invasión Jesús Usón

The inflammation mechanisms in myocardial infarction are altered after intrapericardial administration of cardiosphere-derived stem cells and their extracellular vesicles.

# P689

**Esther López** Centro de Cirugía de Mínima Invasión Jesús Usón

Intrapericardial administration of Cardiosphere Derived Cells early after experimental myocardial infarction in swine: safe, easy but of limited effectiveness.

#### P691

**Tobias Wimmer** *Justus-Liebiq University* Giessen

A bioluminescence resonance energy transfer based sensor for the precise determination of non-homologous end joining DNA repair events

## P693

Anastasia Efimenko Lomonosov Moscow State University

Mesenchymal stromal cell secretome as a promising tool for male infertility treatment

#### P695

Chao-Liang Wu National Cheng Kung University Medical College

Silencing of prothymosin  $\alpha$  as a therapeutic strategy for the treatment of polycystic kidney disease

## P697

**Estefania Rodriguez** CIMA, Universidad de Navarra

AdrA as a potential immunomodulatory candidate for STING-mediated anti-viral therapy

## P699

**Seung Bin Yoon** *Korea Research Institute of* Bioscience & Biotechnology (KRIBB) Reference values of haematological and biochemical parameters in young-adult cynomolgus monkey (Macaca fascicularis) and rhesus monkeys (Macaca mulatta) nesthetized with ketamine hydrochloride

# P701

Regina Kashapova Kazan Federal University Creation of technology for manufacturing composite titanium polymer implants

# P703

**Yi Liu** Capital Medical University Low-level laser enhances gingival wound healing through promoting migration of human gingival mesenchymal stem cells via ROS/JNK/NF-ĸB/MMP-1 pathway





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# Poster session II: Thursday 24 October



# **AAV vector development**

## P002

Kalyani Nambiar University of Pennsylvania Perelman School of Medicine, Philadelphia Transduction evaluation of novel AAV natural isolates in nonhuman primates using a barcoded transgene system

#### P004

Diana Cepeda Adverum Biotechnologies Inc., Menlo Park, California, USA

*In vivo* screening of an adeno-associated virus capsid library in non-human primate eyes identifies a novel AAV variant with superior retinal penetration and transduction by intravitreal delivery

## P006

Marti Cabanes-Creus Children's Medical Research Institute

Modulating the heparan sulfate proteoglycan binding of AAV2 enhances in vivo transduction of human hepatocytes in a xenograft mouse model

## P008

Waldemar Schäfer University Medical Center Hamburg-Eppendorf

Nanobody-enhanced targeting of AAV vectors

# P010

Claus Hallwirth Children's Medical Research Institute

Nature trumps nurture: Use of naturally livertropic adeno-associated viruses as vector platforms for liver-directed human gene therapy

#### P012

**MatthewFuller** *Ultragenyx Pharmaceutical* Characterization of HPV-related impurities in HeLa producer cell line generated rAAV and in vivo preclinical analysis of HPV-related host cell DNA persistence and gene expression following vector delivery

#### P014

Simon Chanas GlaxoSmithKline

Over-expression of the E. coli single stranded DNA binding protein (ssb) inhibits the loss of Adeno-associated virus (AAV) hairpin loop inverted terminal repeats in transfer vector plasmid preps

#### P016

**Hong You** Beijing Friendship Hospital, Capital Medical University

Casein kinase 2 phosphorylates Rep78 of adeno-associated virus (AAV) type 2 Rep78 with effects upon its biochemistry and boosts AAV replication

#### P018

Nicholas Buss REGENXBIO Inc.

AAV9.hCLN2 (RGX-181) improves survival and neuropathology in TPP1m1j mice, a model for CLN2 Batten disease

#### P020

**Elena lurlova** *Biotechnology company* BIOCAD, Saint-Petersburg, Russia Optimization of elution conditions for immunoaffinity chromatography of AAV5 and AAV9 vectors





Axel Rossi Hannover Medical School. Hannover, Germany

Capsid engineering overcomes barriers toward Adeno-associated viral (AAV) vectormediated transduction of endothelial cells

# P024

Mathieu Mevel INSERM UMR 1089, University of Nantes, CHU de Nantes, Nantes Chemically modified AAV vectors for gene therapy

## P026

Esther Attebi Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE Development of purification step for several AAV serotypes using POROS™ CaptureSelect™ AAVX affinity chromatography

# P028

Roman Raim Baxalta, a Takeda company

Critical aspects in implementing a precise and reliable droplet digital PCR method as dose measurement assay for determining vector genomes in rAAV- based Gene Therapy products

#### P030

**Huseiyin Besir** *PROGEN Biotechnik* GmbH

The new AAV3 Titration ELISA - continued tradition of reliable AAV titer determination

## P032

**Akihiro Kume** *Jichi Medical University* Adeno-associated virus (AAV) serotype 3-based vector as an alternative vehicle for liver-directed gene therapy

# P034

Eva Reinauer Leukocare AG

Effective stabilization of viral vectors in liquid using an algorithm-based development approach

## P036

Nihay Laham Karam A.I. Virtanen Institute, University of Eastern Finland Enhanced Adeno-associated virus transduction in endothelial cells

# **Viral vectors manufacturing**

#### P040

WeihengSu University of Oxford Self-repressing 'helper' adenovirus enabled efficient manufacture of adeno-associated viral vectors without contamination by adenovirus or small drugs

# P042

**Lorella Tripodi** SEMM-European School for Molecular Medicine, Napoli, Italy Synergistic effect of combined administration of an oncolytic adenovirus and Bifidobacterium spp. supplements in a mouse model of melanoma

#### P044

Martin Busch Heidelberg University Hospital Impact of production impurities on potency of Adeno-associated virus vectors: Systematic comparison of density gradient and affinity purification

# P046

Eduard Ayuso INSERM UMR1089, University of Nantes, CHU de Nantes, Nantes Automated HEK293 suspension cell adaptation to new media and use of new DoE approach to optimize AAV production in suspension utilizing the ambr®15 platform

#### P048

Qian Liu Oxford Genetics Ltd Scalable and high-titre lentiviral vector production from cGMP-compliant clonal suspension HEK293 cell line

## P050

**Eric Faulkner** Homology Medicines Inc Impact of full and empty particle concentration on product quality and in vivo efficacy of HMI-102 in a mouse model of phenylketonuria

## P052

Pranav Joshi McGill University, Quebec, Canada

Chromatographic approach for separation of AAV capsid variants

## P054

Isobel Searing Oxford BioMedica Analytical development and automation of lentiviral vectors

#### P056

Joseph Zeguer Orchard Therapeutics PLC A BAC-cloning platform for development of stable producer cell lines for commercial scale lentiviral vector manufacture

## P058

**Dina Glazkova** Center for Strategic planning and management of medical and biological risks, Russian Federation

Interposition of genes in anti-HIV lentiviral vector affects its titer and antiviral activity

#### P060

**Jian Gao** *Witten/Herdecke University* Improved delivery of tumor specific oncolytic adenovirus type 5 with NK-92 carrier cells in cancer treatment

## P062

Thomas Williams Oxford BioMedica Industrial Lentiviral vector manufacturing using advanced process analytical technologies

# P064

**Ana S. Coroadinha** *iBET, Instituto de* Biologia Experimental e Tecnológica, Oeiras, **Portugal** 

Enabling stable lentiviral vector producer cell line generation: Identifying barriers to high productivities





Man Sub Kim Osong Medical Innovation Foundation

Application of the design of experiment method for AAV vector production in suspension cells

# P068

**Cristina Salado Manzano** *University of* Barcelona, Barcelona, Spain Innovative cell-based therapy to treat Huntington's Disease

## P070

# Marie Jourdan Univercells

Scalable single-use technology to meet gene therapy & vaccines production demands

# P072

Annu Luostarinen Research & Development, Finnish Red Cross Blood Service, Helsinki, **Finland** 

Comparative analysis of lentiviral vector final formulation conditions for the ex vivo transduction of primary human T-cells

#### P074

# **Eva Fong** Merck Millipore

Development and scale-up of a bench-scale bioreactor process for transient lentivirus production using a suspension-Adapted HEK293T clone

#### P076

Juan Carlos Ramirez VIVEbiotech

SerLess: An adherent HEK293-derived producer cell line for manufacturing lentiviral vectors in the absence of animal-derived serum

# P078

**Emmanuelle Cameau** PALL Biotech

Cost modeling comparison of static, suspension and fixed-bed bioreactors to manufacture commercial gene therapy products

## P080

## Matt Krusen Aldevron

Advantages of standardization and large-scale manufacturing of helper plasmids for viral vector production

# P082

Joëlle Cheuzeville Genethon, UMR \$951, Inserm, Univ Evry, Université Paris Saclay, **EPHE** 

Improvement of lentivirus manufacturing in large-scale bioreactors using PTG1plus, a novel cationic transfection agent, in suspension HEK 293T cells

## P084

**Miguel Chillon** *Universitat Autònoma de* Barcelona (UAB), Bellaterra, Spain UPV: Production of customized high quality viral vectors at the joint UAB-VHIR technological platform

#### P086

Ram Shankar PlasmidFactory GmbH & Co. KG RNase-PF: An animal-free recombinant solution for the removal of host-cell RNA

# **Cancer**

# P090

Els Verhoeyen EVIR/CIRI/INSERM U1111, Lvon

Efficient and robust NK-cell transduction with baboon envelope pseudotyped lentivector: A new tool for CAR NK cell immunotherapy

## P092

Simona Porcellini MolMed S.p.A GMP grade CD44v6 CAR-engineered T cells control tumor growth in lung and ovary adenocarcinoma bearing mice

#### P094

**Katharina Bergerhoff** *GammaDelta Therapeutics* 

Human Vδ1+ T cells; an allogenic 'off-the shelf' T cell therapy platform

# P096

**Leysan Minigulova** *Kazan Federal University,* Kazan, Russia

Transmembrane topogenesis and folding of extramembrane domains of NaPi2b transporter under conditions of malignant transformation can lead to the appearance of tumor-specific epitopes

#### P098

Julia Davydova University of

Pigs versus rodent models for assessing performance of serotype chimeric Ad5/3 oncolytic adenoviruses

# P100

Juan José Rojas Expósito Ludwig-Maximilians-Universitat München Induction of immunogenic tumor cell death by a novel immune-oncolytic Vaccinia virus strain leads to robust antitumor immune responses directed against neo-epitopes

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# Estela Núñez Manchón IDIBAPS

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Feasibility of gene therapy for Friedreich Ataxia-associated cardiomyopathy in nonhuman primates: Evaluation of delivery route, biodistribution and expression following AAVRh.10.FXN administration

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Sergio Antonio Munoz CBATEG-UAB

A new rat model of Pompe disease generated by CRISPR/Cas9 technology mimics the human disease phenotype

# P484

Astrid Brull Sorbonne Université, Inserm UMRS 974, Institut de Myologie

Wild-type lamin A overexpression combined with mutant Lmna knock-down extends lifespan in a murine model of LMNAcongenital muscular dystrophy.

#### P486

**Capucine Trollet** INSERM U974 Sorbonne Université Institut de Myologie Transduction efficiency of AAV serotypes after local injection in mouse and human skeletal muscle

#### P488

**Eric Ehrke-Schulz** Witten/Herdecke University, Witten, Germany

CRISPR/Cas9 mediated gene editing of Duchenne muscular dystrophy with a single gene deleted adenoviral vector

#### P490

**Zoya Tsokolaeva** V.A.Negovsky Research Institute of General Reanimatology Transplantation of scf-expressing mesenchymal cell sheet activates epicardium and attenuates adverse cardiac remodeling in acute myocardial infarction

## P492

Artur Fedianin Kazan Federal University, Kazan

Investigation of the mechanisms of reorganization of motor control in the period of postgravitational readaptation

#### P494

Cynthia Daoud Genethon, UMR\_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE A new rat model for Duchenne muscular dystrophy generated by CRISPR-induced deletion of Exon 45

# P496

**Zhipeng Fan** Capital Medical University School of Stomatoloav

The CB1 enhanced the osteogenic differentiation ability of mesenchymal stem cells in the inflammatory environment

## P498

**Sang Han** Federal University of Sao Paulo IL-4 gene overexpression promotes repair of the ischemic skeletal muscle

# P500

**Margarita Codinach** Banc de Sang i Teixits

Impact of nucleated cell isolation method on the manufacturing of mesenchymal stromal cell-based medicinal product

#### P502

Sandra Fuertes Biodonostia Institute Structural analysis of neuromuscular junctions in aging: Effect of Sox2 heterozygosity

# P504

Ilnur Ishmukhametov Kazan Federal University

Influence of species-specific plasmid pBUDKecVEGF164-ecBMP2 on osteogenesis MSC horse in vitro

#### P506

**Denis Buev** Ryazan State Medical University named after academician I.P. Pavlov Ouantitative index of cell fusion for cell therapy development

## P508

Alexander van den Boom Witten/Herdecke University, Witten

Cell based screening of different adenoviral serotypes to identify candidates for the development of improved vectors for the treatment of muscular disorders

## P510

Ilnur Ishmukhametov Kazan Federal University

Chitosan/gelatine/agarose nanostructured scaffolds promote angiogenesis

# P512

Maxim Baltin Kazan Federal University

Introduction of mesenchymal stem cells reduced the effect of ischemia on nitric oxide content in the hippocampus and restored the approximate motor activity of rats after modeling of cerebral stroke

# P514

Elvira Akhmetzyanova Kazan Federal University

Novel rat model of hindlimb ischemia for assessment of the efficiency of gene and cell therapy

# Poster session II: Thursday 24 October



#### P516

Luis López Navas Andalusian Network for Design and Translation of Advanced **Therapies** 

Functional characterization of a proprietary GMP human platelet lysate

# P518

Elvira Akhmetzyanova Kazan Federal University

Vegf and fgf2 genes stabilizes angiogenic effect in hind limb ischemia model in rats

# P520

**Artur Fedianin** Kazan Federal University Influence of unilateral deforming arthrosis on the condition of contralateral limb

#### P524

Margarita Codinach Banc de Sang i Teixits New uses for existing technologies: Automated enumeration of mesenchymal stem cells with haematology analysers.

## P526

**Yu Cao** Beijing Key Laboratory of Tooth Regeneration and Function Reconstruction

Effect of secreted frizzled-related protein2 on regeneration of dental pulp stem cells

# Skin gene and cell therapy

## P530

**Oliver March** Paracelsus Medical University Salzburg, Salzburg

Highly efficient paired nickase-mediated correction of junctional epidermolysis bullosa via COL17A1 reframing

## P532

Lucía Martínez-Santamaría CIEMAT/CIBERER. Madrid

Safety criteria and patient selection in a cellbased clinical trial for the systemic treatment of recessive dystrophic epidermolysis bullosa: MesenSistem-EB

## P534

**Ana Cardesa Gil** Andalusian Network for design and Translation of Advanced **Therapies** 

Compassionate use of mesenchymal stromal cells for the treatment of graft-versus-host disease

## P536

Jose Bonafont Carlos III University (UC3M), Madrid

Highly efficient gene-editing strategies for clinically-relevant ex vivo correction of recessive dystrophic epidermolysis bullosa in primary patient cells

#### P538

Hyung-Sik Kim Pusan National University

Superoxide dismutase 3-introduced stem cells and their extracellular vesicles exert improved efficacy in the murine dermatitis model

#### P540

**Thomas Kocher** Paracelsus Medical University Salzburg, Salzburg

A selection-free COL7A1 repair strategy based on homologous recombination

# P542

Laura Yndriago Biodonostia Institute Characterization of aged dermal stem cell phenotype: implications for skin homeostasis

# P544

Regina Kashapova Kazan Federal University, Engineering Institute

Development of matrixes and devices on the basis of inert materials by the method of ion-beam treatment to prototype cells and medical targets

# New approaches in gene editina

# P550

**Lili Wang** *University of Pennsylvania* In vivo genome editing of hAPOC3 in the liver of APOC3 transgenic mouse leads to a robust and stable reduction in serum triglyceride levels and normalization of lipid profiles

#### P552

Sean Burns Intellia Therapeutics CRISPR/Cas9-mediated gene knockout to address primary hyperoxaluria

#### P554

Maria Silvia Roman Azcona Institute for Transfusion Medicine and Gene Therapy

A versatile reporter system to identify designer epigenome modifiers for effective multiplexed epigenome editing

# P556

Giulia Maule University of Trento - CIBIO Allele specific repair of splicing mutations in cystic fibrosis through AsCas12a genome editing

## P558

Kunwoo Lee GenFdit

Efficient delivery of mRNA and CRISPR in CNS using polymer nanoparticle

#### P560

Juan Roberto Rodriguez Madoz

Regenerative Medicine Program, CIMA Universidad de Navarra In vivo CRIPSR/Cas9 Ldha inhibition as universal treatment for primary hyperoxaluria

#### P564

Christina Fuentes University of California, Berkelev

An engineered self-inactivating adenoassociated virus mediated Cas9 delivery system for therapeutic genome editing





Julia Fakhiri Heidelberg University Hospital

Kill-switch and self-inactivating AAV vectors based on CRISPR/Cas9 for precise control of in vivo gene expression

# P568

**Estel Aparicio Prat** Simulation and Modeling Sciences, Pfizer, Cambridge

Using CRISPR base editors to perform targeted mutagenesis screenings for mechanism of action discovery

#### P570

**Teresa Rojo Romanos** *TU Dresden* Designer recombinase for precise excision of HTLV-1

## P572

**Omer Anakok** Ataturk University Direct transgene expression and mAb production by using new generation minimised UCOEs on human iPS and CHO cells

#### P574

François Moreau-Gaudry INSERM U1035 Universite Bordeaux CRISPR-Cas9 genome editing induces megabase-scale deletions

## **P578**

Francesca Tasca Leiden University Medical Centre

High-capacity adenoviral vectors encoding full-length dystrophin and CRISPR-Cas9 nucleases for targeted correction of myogenic cells from Duchenne muscular dystrophy patients

#### P580

Aida Garcia Torralba CIEMAT/CIBERER. Madrid

Efficient generation of knock-out mice by in vitro and in vivo electroporation of CRISPR/ Cas9 system to model rare metabolic inherited diseases and cancer predisposition

#### P582

Álex Jiménez Félix University of Barcelona

Gene therapy by correction of point mutations in the endogenous locus of mammalian cells using Repair-PolyPurine Reverse Hoogsteen hairpins

#### P584

Cristina Rocha AstraZeneca. Biopharmaceutical R&D

Defining safety margins of therapeutic genome editing by understanding mechanisms and liabilities of CRISPR-based molecular entities

#### P586

Sabina Sánchez Hernández GENyO-Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia

Development of cellular models to study efficiency and safety of gene repair in Wiskott-Aldrich syndrome

#### P588

**Jeongpil Han** Seoul National University Cell cycle determines efficiency of homology directed repair depending on the integrity of nuclear envelope

#### P590

**Irina Starostina** *Kazan Federal University* Gene modification of HEK293A cells using CRISPR-Cas9 SAM technology for the transcriptional activation of dysferlin gene

# P592

**Takahiro Nakamura** Kyushu University Designer RNA binding protein based on PPR protein, as a new modality for targeted therapy

## P594

Andrew Olsen Aldevron

Aldevron's CRISPR-associated nucleases for gene editing: Tools to support discovery and therapeutic programs

# **New delivery systems**

## P600

Antonia Follenzi Universita del Piemonte Orientale

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

# P602

María Sancho CIBER-BBN

Combination of exosomes and near-infrared responsive gold nanoparticles: New selective and specific therapeutic vehicle.

#### P604

Gaelle Vandermeulen University of Louvain DNA immunization against tumor epitopes using a versatile delivery platform that exploits inherent mechanisms of antiviral defense

# P606

**Francesco Piras** *SR-TIGET, Milan* Exploring combinatorial relief of multiple innate immune blocks for efficient gene engineering of guiescent human haematopoietic stem cells

## P608

Pilar Puig Serra Centro Nacional de Investigaciones Oncológicas Development of virus-like particles for CRISPR/ Cas9 targeted delivery

# P610

Ya-Fang Mei Institute of Clinical Microbiology, Virology, Umeå University

Transcriptome analysis of metastatic prostate cells infected by oncolytic adenovirus 11p encoded ADP gene (RCAd11pADP) expose important antitumor pathways

# P612

Mahito Nakanishi National Institute of Advanced Industrial Science and Technology

Development of stealth RNA vector (SRV) for innovative gene and cell therapy





**Eirini Vamva** *University of Cambridge* Towards optimising lentiviral vectors for gene therapy through structure informed genome modification

# P616

Tomoyuki Nishikawa Osaka University Development of antitumor immunity inducing RNA drug and a novel pyro-drive jet injector

#### P618

Ekaterina Slobodkina Faculty of Medicine, Lomonosov Moscow State University Combined plasmid delivery of angiogenic growth factors: Preclinical assessment of different approaches

#### P620

Lorena de Oñate UC Berkeley Delivery of an endosomolytic CRISPR-Cas9 RNP via receptor-mediated endocytosis for cell-targeted genome editing

#### P622

**Chuanyu Zhuang** *Hanyang University* RAGE-targeting delivery of anti-microRNA-92a antagomir for the treatment of acute lung injury

#### P624

**Chin Yang Chang** Osaka University Development of novel injector: Pyro-Drive Jet Injector (PJI) application to intradermal DNA vaccination

#### P626

Sirina Kurbangaleeva Kazan Federal

Mesenchymal stem cells derived membrane vesicles induced by cytochalasin B demonstrate immunomodulatory properties

# P628

**Natalia Bal** Institute of Higher Nervous Activity and Neurophysiology of RAS, Moscow Effect of intranasal administration of mesenchymal stem cells on the average speed of movement and time of activity and NO level in olfactory bulb of rats after simulation of brain stroke

#### P630

Keita Takahashi Gifu Pharmaceutical University

Identification of cellular targets for DNA delivery from recombinant L. lactis strains in vivo

# P632

**Hiu Man Grisch** *University Children's Hospital* Zurich and Children's Research Center Fusion-dependent formation of lipid nanoparticles containing macromolecular payloads

#### P634

Olga Neustroeva Kazan Federal University

Cytokine profile and immunophenotype of cytochalasin B induced membrane vesicles

#### P636

**Geraldine Peyrou** Polyplus Transfection Non-viral in vivo mRNA delivery for cancer research, vaccination or gene therapy

## P638

**Junkyu Ha** *Hanyang University* A fusion peptide with an LPS-binding peptide and a RAGE-binding peptide as a carrier of plasmid DNA with anti-inflammatory effects

#### P640

**Ilnur Ishmukhametov** *Kazan Federal* University

Assessment of toxicity of planar shaped nanomaterials using flow cytometry

#### P642

**Virginia Arechavala** Biocruces Bizkaia Health Research Institute

A COST network to improve the delivery of antisense RNA therapeutics

## P644

Myoungjee Choi Hanyang University A mixed micelle of His-Arg conjugated PAMAM G2 with cholesterol and Glycyrrhizic acid as gene delivery system into the lung cells

## P646

Ilnur Ishmukhametov Kazan Federal University

Encapsulation of E. coli bacteria cells with polyelectrolytes as a method of obtaining microcapsules for targeted drug delivery

#### P648

Yulia Yuzefovych Hannover Medical School Generation of immunologically invisible transgenic porcine pancreatic islet cell clusters after single cell engineering and islet reassembling to support xenograft survival

#### P650

Ilnur Ishmukhametov Kazan Federal University

Hyperspectral microscopy in the characterization of aluminosilicate halloysite nanocontainers

# **Regulatory session**

#### P656

Roke Iñaki Oruezabal Guijarro Andalusian Network for Design And Traslation of Advanced **Therapies** 

Regulatory framework for innovation assessment in advanced therapies in Europe

# P658

**Myriam Lemmens** Novartis Pharma AG Transformation potential of CRIPSR/Cas9 based genome editing can be assessed by soft agar colony formation assay and growth in low attachment plates

## P660

**Álvaro Ritoré Hidalgo** *Andalusian Network* for Design and Translation of Advanced **Therapies** 

Public healthcare system as key player in leveraging advanced therapy supply chains





**Diane Seimetz** Biopharma Excellence ATMPs at the transition from nonclinical to clinical stage: How to smartly mitigate risk and uncertainties

# P664

**Elena Meurer** Biopharma Excellence Preserve and recreate: Manufacturing and quality development for tissue, gene and cell therapy products

## P666

**Florian Durst** Thermo Fisher Scientific A rapid alternative to culture based mycoplasma detection

#### P668

**Liliya Chernova** *Kazan Federal University* Nontrivial properties of small heat shock protein IbpA from Acholeplasma laidlawii

# Other

#### P670

Jessika Ceiler NCT/DKFZ, Heidelberg Role of Mitochondria-Associated Membranes in recombinant AAV mitochondrial integration and trafficking

## **P672**

Mercedes Lopez-Santalla CIEMAT/CIBERER, Madrid

Long-term protective effects of adiposederived mesenchymal stromal cell therapy in experimental colitis

## P674

**Fabiola Lora Ulgar** *Andalusian Network* for Design and Translation of Advanced **Therapies** 

Safety, feasibility and trends of efficacy of intravenous injection of autologous adiposederived mesenchymal stromal cells in patients with amyotrophic lateral sclerosis: A phase I-IIa multicenter randomized triple blind placebo controlled trial

## P676

Rocio Sampayo University of California, Berkeley

Mechanical tuning of adult hippocampal neural stem cells using light-responsive Rho GTPases dictates cell fate

#### P678

Adriano de Marino SR-TIGET, Milan ISwap: a bioinformatics tool for index switching detection in vector integration site studies

#### P680

Sandra Laner-Plambeger Department for Transfusion Medicine, Paracelsus Medical University Salzburg

Upregulation of mitotic bookmarking transcription factors may induce enhanced proliferation of human stromal cells by human platelet lysate

#### P682

Anastasia Efimenko Lomonosov Moscow State University

Tissue-specific interaction of stromal cells and endothelium in vitro leads to a distinct pattern of angiogenic response

#### P684

Nuria Nieto-Nicolau Barcelona Tissue Bank & donor center

Good manufacturing production of limbal stem cells in a tissue bank for clinical application

# P686

**Esther López** Centro de Cirugía de Mínima Invasión Jesús Usón

Efficacy of intracoronary administration of microencapsulated hepatocyte growth factor in a reperfused myocardial infarction swine model

## P688

**Esther López** Centro de Ciruaía de Mínima Invasión Jesús Usón

Effects of early intrapericardial delivery of microvesicles obtained from heart-derived cells in cardiac function in an experimental myocardial infarction in swine.

#### P690

Roke Iñaki Oruezabal-Guijarro Andalusian Network for Design And Traslation of Advanced **Therapies** 

External quality control program in advanced therapies

#### P692

Nuria Nieto-Nicolau Barcelona Tissue Bank & donor center

Integrin a6 improves stemness, proliferation and migration in human mesenchymal stem cells

#### P694

Raquel Fernández Pérez CIEMAT/CIBERER, Madrid

Role play by galectin-1 in regulatory CD4+ T cell therapy for modulation of immune responses in experimental colitis

# P696

**Seung-Bin Yoon** *Korea Research Institute of* Bioscience and Biotechnology (KRIBB) Quantitative analysis of spliced X-box binding protein 1 (XBP1) using universal primer for quantitative RT-PCR

## P698

**Ruth Rieser** *Ludwig-Maximilians-Universitat* München

Effect of salt and sugar on the storage stability of liquid and freeze-dried adeno-associated virus formulations

#### P700

**Lucia Dunajova** *Synthace Ltd, London* Antha – a powerful software solution for the automated laboratory of the future

#### P702

**Paul Foulds** *Cell Therapy Catapult* Leaders in cryogenic RFID technology

# P704

Regina Kashapova Kazan Federal University, Engineering Institute Electromyographic analysis of the injured limb