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FILTER BY CATEGORY

AVV IMMUNOLOGY				
No.	**Category**	**Speaker**	**Speaker Affiliation**	**Poster Title**
P001	AAV immunology	N Zabaleta	*Mass Eye and Ear/Harvard Medical School*	**An AAV-based, room-temperature stable, single dose COVID-19 vaccine**

P002	AAV immunology	M L Couce Pico	*Hospital Clínico Universitario de Santiago de Compostela, Santiago de Compostela, Spain*	**Long-term, sustained efficacy and safety from a phase 1/2 clinical trial of an AAV8-mediated liver-directed gene therapy in adults with glycogen storage disease type Ia**
P003	AAV immunology	S S Leung	*Selecta Biosciences*	**Enhanced level and durability of AAV transgene expression and mitigation of anti-capsid neutralizing antibodies by ImmTOR tolerogenic nanoparticles in nonhuman primates**
P004	AAV immunology	I Ros-Gañán	*Vivet Therapeutics S.L.*	**Optimizing IdeS treatment regimen for enhanced AAV transduction in individuals with pre-existing anti-AAV neutralizing antibodies**
P005	AAV immunology	J Lemoine	*Université Paris-Saclay, Univ Evry, Inserm, Généthon, Integrare research unit UMR_S951, 91000, Evry-Courcouronnes, France*	**Novel AAV capsid variant for muscle-directed gene therapy**
P006	AAV immunology	S Babutzka	*Department of Ophthalmology, University Hospital, LMU Munich, Munich, Germany*	**Engineered AAV-based vaccines against SARS-CoV-2**
P007	AAV immunology	C Morival	*Inserm U1089*	**rAAV sensing in microglial cells**
P008	AAV immunology	J M Alexander	*Spark Therapeutics*	**The Endopeptidase IdeS Degrades Anti-AAV IgG and Enables Liver-Directed AAV Gene Therapy in Animal Models of Humoral Immunity**
P009	AAV immunology	J Rodó	*Svar Life Science*	**Characterization of a reporter gene cell line for the easy assessment of therapeutic AAV vectors – a case study**
P010	AAV im munology	P O Ilyinskii	*Selecta Biosciences*	**Efficient suppression of IgG antibody responses to high doses of AAV8 capsids by single and multiple administrations of ImmTOR nanoparticles**
P011	AAV immunology	P O Ilyinskii	*Selecta Biosciences*	**ImmTOR combined with B cell-targeted therapies provides synergistic activity in mitigating anti-AAV capsid antibody responses and enables repeated vector dosing**
P012	AAV immunology	K Lau	*BioMarin Pharmaceutical*	**Evaluation of prophylactic immune suppression and application to repeat dose administration of AAV-mediated gene therapy in mice**
P013	AAV immunology	J Haar	*Research Beyond Borders, Boehringer Ingelheim Pharma GmbH & Co. KG, Biberach an der Riss, Germany*	**Novel MSD-based screening assays to quantify pre-existing antibodies against various AAV serotypes**
P014	AAV immunology	T Klem	*Homology Medicines, Inc.*	**Neutralizing antibody prevalence toward a hematopoietic stem cell-derived AAV and immunoassays for clinical trial enrollment**
P015	AAV immunology	J S Diallo	*Virica Biotech*	**Increasing Gene Therapy Vector production using Viral Sensitizer Molecules**

P016	AAV immunology	M Ertelt	*Institute for Drug Discovery, University Leipzig Medical School, Leipzig 04109, Germany*	**Change in capsid-HSPG interaction through peptide insertion in variable loop VIII in liver-targeting AAV vectors**

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CANCER GENE THERAPY

No.	Category	Speaker	Speaker Affiliation	Poster Title
P017	Cancer Gene Therapy	F Rossari	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Interferon-α gene delivery by tumor-associated macrophages improves function and prevents exhaustion of B7-H3-redirected CAR T cells in glioblastoma**
P018	Cancer Gene Therapy	A Potenza	*San Raffaele Scientific Institute*	**CRISPR/Cas9-mediated CD39 disruption can be combined with TCR editing in T cells to improve the adoptive T cell therapy of colorectal cancer**
P019	Cancer Gene Therapy	C Ekentok-Atici	*Pharmaceutical Biotechnology Department, Marmara University, Istanbul, 34854, Turkey*	**Administration of Ad5/chitosan/PEG-aptamer vector delivering PDGF-D shRNA decreases tumor growth in rat breast cancer model**
P020	Cancer Gene Therapy	A Franke	*Hannover Medical School*	**Towards a single-shot prime-boost AAV-based vaccine for cancer immunotherapy**
P021	Cancer Gene Therapy	T Kerzel	*San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)*	**In-vivo gene-based immune reprogramming of liver metastases enables protective T cell responses**
P022	Cancer Gene Therapy	M Martinez-Lage	*Centro Nacional de Investigaciones Oncológicas*	**CRISPR-mediated targeting of fusion oncogenes in combination with chemotherapy enhances the efficacy of cancer cells elimination and tumor growth reduction in xenograft models.**
P023	Cancer Gene Therapy	N G Tessarollo	*University of São Paulo School of Medicine*	**Membrane coating for nonreplicating adenoviral vectors aids gene delivery in a murine model of melanoma**
P024	Cancer Gene Therapy	C Moore-Kelly	*Oxford BioMedica*	**Prostratin as a small molecule inducing agent for improving lentiviral vector titres.**
P025	Cancer Gene Therapy	T Briolay	*Université de Nantes*	**Development of a bio-inspired nanovector for targeted cancer gene therapy**
P026	Cancer Gene Therapy	R Chan	*Bio-Techne*	**Assessment of Natural Killer (NK) cell activity for immunotherapy using a novel flow cytometry-based killing assay**

P027	Cancer Gene Therapy	H Bareke	*Marmara University, Istanbul*	**Actively targeted chitosan-based vector for efficient CRISPR-Cas9 mediated Tenascin-C gene editing in triple negative breast cancer**
P028	Cancer Gene Therapy	C A Tavira-Montalvan	*Pharmaceutical Biotechnology Laboratory. Faculty of Pharmacy. Autonomous University of Morelos, Morelos, México.*	**Evaluation of transduced cultures of MCF-7 cells with a first-generation adenoviral vector expressing the caspase-3 gene as a model for breast cáncer.**

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CAR T HAEMATOLOGICAL

No.	Category	Speaker	Speaker Affiliation	Poster Title
P029	CAR T Haematological	C Calviño	*Area de Terapia Celular, Clinica Universidad de Navarra. IdiSNA. Pamplona, 31008, Spain.*	**Transcriptional and functional assessment of CAR-T cells targeting CD33 from AML patients and healthy donors.**
P030	CAR T Haematological	M Fredon	*UMR1098-RIGHT/EFS BFC/UFC, Besançon, 25000, France*	**Optimization of the CD123 CAR T-cell scFv for the treatment of patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)**
P031	CAR T Haematological	C Bauche	*Ixaka*	**Development of in vivo anti-CD19 CAR T-cell therapy based on chemically encapsulated lentiviral vectors**
P032	CAR T Haematological	P Rodríguez-Márquez	*Hemato-Oncology Program. CIMA Universidad de Navarra. IdiSNA. Pamplona, 31008. Spain.*	**CAR density influences CAR-T antitumoral efficacy and correlates with clinical outcome**
P033	CAR T Haematological	V Picanço-Castro	*Center for Cell-based Therapy (CTC), Regional Blood Center of Ribeirão Preto, University of São Paulo, Ribeirão Preto, São Paulo, Brazil.*	**Development of T-CAR and NK-CAR using a nonviral, nonintegrating, episomal DNA vector**

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CARDIOVASCULAR & LUNG DISEASES

No.	Category	Speaker	Speaker Affiliation	Poster Title
P034	Cardiovascular & lung diseases	C I Juarez-Molina†	*Imperial College London*	**Lentiviral vector/GM-CSF Gene Therapy for Autoimmune Pulmonary Alveolar Proteinosis**
P035	Cardiovascular & lung diseases	R Maeshima	*University College London*	**Development of in vitro transcribed mRNA therapeutics for cystic fibrosis**

P036	Cardiovascular & lung diseases	A Moiseenko	*Boehringer-Ingelheim Pharma GmbH & Co. KG*	**Functional characterisation of an engineered next generation lentivirus vector for the treatment of cystic fibrosis**
P037	Cardiovascular & lung diseases	J W Hickmott	*Imperial College London*	**Single cell tools for measuring mRNA and protein expression for gene therapy**
P038	Cardiovascular & lung diseases	J W Hickmott	*Imperial College London*	**Modifying signal peptides for respiratory gene therapy with secreted proteins**
P039	Cardiovascular & lung diseases	R V Bell	*Imperial College London*	**Regulation of lentivirus-mediated expression in a human airway model**
P040	Cardiovascular & lung diseases	S Artusi	*Krystal Biotech, Inc.*	**Preclinical pharmacology of KB408, an HSV-1-based gene therapy vector, for the treatment of alpha-1 antitrypsin deficiency**
P041	Cardiovascular & lung diseases	M Á de Pedro	*Jesús Usón Minimally Invasive Surgery Centre, Cáceres, Spain*	**Proteomic changes induced by intrapericardial administration of secretomes from menstrual blood stromal cells on the infarcted areas of porcine myocardium**
P042	Cardiovascular & lung diseases	S Bobis-Wozowicz	*Jagiellonian University*	**Hypoxic extracellular vesicles protect human cardiomyocytes from oxidative damage**
P043	Cardiovascular & lung diseases	A Bettini	*University College London*	**Live-tracking of biomaterial distribution using CT-visible microspheres after non-invasive delivery into the heart**
P044	Cardiovascular & lung diseases	C Rodríguez-González	*Clinic for Pediatric Pneumology, Allergology and Neonatology, Hannover Medical School, Hannover, Germany*	**iPSC-derived macrophages from Cystic Fibrosis patients for infection models and drug screening platforms.**
P045	Cardiovascular & lung diseases	S M Quinn	*Imperial College London*	**FGF7 treatment improves survival and proliferation of injured primary lung epithelial cells**
P046	Cardiovascular & lung diseases	M Á de Pedro	*Jesús Usón Minimally Invasive Surgery Centre, Cáceres, Spain*	**Alleviated inflammatory response in infarcted tissue after intrapericardial administration of secretome of prime menstrual blood stromal cells**
P048	Cardiovascular & lung diseases	Y Lyu	*Labor Molekulare Kardiologie - UKSH*	**Krüppel-Like Factor 4 as a novel gene therapy target contributes to Hypoxic Pulmonary Hypertension**
P049	Cardiovascular & lung diseases	Z H Yang	*Tenaya Therapeutics*	**Cardiac AAV:PKP2 gene transfer prevents development of arrhythmogenic cardiomyopathy in a PKP2-deficient mouse model**

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CNS & SENSORY

No.	Category	Speaker	Speaker Affiliation	Poster Title
P050	CNS & sensory	T Schilling	*PTC Therapeutics, Inc*	**Gene therapy with eladocagene exuparvovec improves cognition and language in patients with aromatic l-amino acid decarboxylase deficiency**
P051	CNS & sensory	T Schilling	*PTC Therapeutics Switzerland, GmBH*	**Eladocagene exuparvovec gene therapy improves motor development in patients with aromatic l-amino acid decarboxylase deficiency**
P052	CNS & sensory	V Zancanella	*uniQure biopharma B.V.*	**Efficacy of C9orf72 ALS gene therapy using miQURE® and widespread distribution in cortical and spinal regions in non-human primates**
P053	CNS & sensory	F Piguet	*NEUROGENCELL, Institut du Cerveau et de la Moelle épinière, INSERM U1127, Hôpital Pitié-Salpêtrière, 47 bd de l'Hôpital, 75013, Paris, France*	**Modulation of brain cholesterol metabolism through CYP46A1 overexpression for Rett syndrome**
P054	CNS & sensory	F Cascino	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Development of chimeric GALC enzymes with improved bioavailability to refine gene therapy strategies for Globoid cell Leukodystrophy.**
P055	CNS & sensory	N Tricaud	*Inserm*	**Local delivery of AAV9 vector in peripheral nerves of mouse, rat and macaque allows a safe, well-tolerated, high and specific delivery to myelin forming cells**
P056	CNS & sensory	V Poletti	*Division of pediatric Hematology, Oncology and Stem Cell Transplantation, Woman's and Child Health Department, University of Padova, Padova, Italy*	**Innovative and Regulated Lentiviral Promoter for the Gene Therapy of Neurodegenerative Diseases**
P057	CNS & sensory	J P Whitton	*Decibel Therapeutics, Inc*	**Minimizing toxicity of AAV-based gene therapy for translation in a pediatric patient population with congenital hearing loss**
P058	CNS & sensory	L M Stanek	*Affinia Therapeutics*	**Anc80L65 results in more widespread gene transfer in the CNS of non-human primates compared to AAV9**
P059	CNS & sensory	V Hernandez-Hernandez	*Institute of Child Health Great Ormond Street - UCL*	**Complete rescue of BBS1 neurometabolic syndrome, brain ventriculomegaly and obesity with a unilateral intracerebroventricular delivery of an AAV9 expressing a codon-optimised BBS1 sequence**
P060	CNS & sensory	E Mangiameli	*HSR TIGET, San Raffaele Telethon Institute for Gene Therapy*	**A human iPSC-based model of Globoid Cell Leukodystrophy uncovers early neurodevelopmental defects.**
P061	CNS & sensory	A P Bemelmans	*MIRCen, CEA*	**Development of an AAV-based model of tauopathy in the mouse visual pathway to study the role of microglia in Tau protein propagation**

P062	CNS & sensory	S Michalakis	*Department of Ophthalmology, University Hospital, LMU Munich, Munich, Germany*	**Preclinical testing of subretinal gene supplementation therapy for PDE6A-linked retinitis pigmentosa**
P063	CNS & sensory	J Gallagher	*University of Massachusetts Medical School*	**From embryo to sheep: Generation of a large animal model of sialidosis**
P064	CNS & sensory	T Schilling	*PTC Therapetuics Switzerland, GmBH*	**Reductions in oculogyric crisis duration and frequency in children with aromatic l-amino acid decarboxylase deficiency treated with eladocagene exuparvovec gene therapy: results from 3 clinical trials**
P065	CNS & sensory	S R Wood	*University of Manchester*	**Sustained, long-term, correction of neuropathology in a mouse model of Hunter Disease following stem cell gene therapy with an LV.IDS.ApoEII vector.**
P066	CNS & sensory	F Piguet	*NeuroGenCell, Institut du Cerveau et de la Moelle épinière, ICM, Inserm U 1127, CNRS UMR 7225, Sorbonne Université, F-75013, Paris, France*	**Complete correction of brain and spinal cord pathology in Metachromatic Leukodystrophy mice**
P067	CNS & sensory	R N Plasschaert	*AVROBIO*	**Transplantation of gene-modified haematopoietic stem cells and their application in murine models of neurodegenerative disease**
P068	CNS & sensory	M M Fernandes Freitas	*Institute of Ophthalmology - UCL*	**Gene therapy results in long-term rescue of photoreceptor function in a mouse model for Bardet-Biedl Syndrome 1**
P069	CNS & sensory	R H Kofoed	*Biological Sciences, Sunnybrook Research Institute, Toronto, Canada*	**Non-invasive AAV delivery to the brain using focused ultrasound and microbubbles**
P070	CNS & sensory	J Ng	*University College London*	**Gene therapy for Dopamine transporter deficiency syndrome: Infantile Parkinsonism-dystonia.**
P071	CNS & sensory	H M Yonutas	*Voyager Therapeutics*	**VY-NPC101: in vitro and in vivo validation of a novel AAV gene replacement therapy to treat Niemann-Pick disease Type C1**
P072	CNS & sensory	L Zobel	*Department of Ophthalmology, University Hospital, LMU Munich*	**In vivo potency testing of subretinal rAAV5.hCNGB1 gene therapy in Cngb1 mouse and dog models of retinitis pigmentosa**
P073	CNS & sensory	S A Broekmans	*uniQure biopharma B.V.*	**Dose dependent lowering of alpha-synuclein and rescue of motor phenotype by miRNA-based AAV gene therapy**
P074	CNS & sensory	R H Porter	*Corlieve Therapeutics SAS, 75008 Paris, France*	**CL002, An AAV9 vector expressing engineered miRNA targeting knockdown of GluK2-containing kainate receptors as a novel gene therapy approach for treating intractable temporal lobe epilepsy**
P075	CNS & sensory	L Smith	*Homology Medicines Inc*	**Blood-brain-barrier crossing leads to long term efficacy in the CNS of HMI-203: Gene therapy development candidate for Mucopolysaccharidosis Type II (MPS II), or Hunter Syndrome**
P076	CNS & sensory	M V Bardina	*Institute of Gene Biology Russian Academy of Sciences, Moscow, Russia*	**AAV-RNAi gene therapy for GNAO1 c.607 G>A encephalopathy in iPSC-derived neurons**
P077	CNS & sensory	Y Ciervo	*Division of pediatric Hematology, Oncology and Stem Cell Transplantation,*	**Development of an ex vivo Gene Therapy for Frontotemporal Dementia (FTD)**

			Woman's and Child Health Department, University of Padova, Padova, Italy	
P078	CNS & sensory	S Spadini	*Division of Pediatric Hematology, Oncology and Stem Cell Transplantation, Woman's and Child Health Department, University of Padova, Padova, Italy*	**Preclinical development of Hematopoietic Stem and Progenitor Cell Gene Therapy for the treatment of Multiple Sclerosis**
P080	CNS & sensory	G Murlidharan	*Voyager Therapeutics*	**Development of CNS-directed GBA1 gene replacement therapy for IV delivery via Blood Brain Barrier Penetrant AAV Capsid**
P081	CNS & sensory	T A Carter	*Voyager Therapeutics*	**Efficient and precise processing of the optimized pri-amiRNA in a Huntingtin-lowering AAV gene therapy: from cultured cells to nonhuman primates**
P082	CNS & sensory	G Devarajan	*Oxford BioMedica*	**OXB-204: A lentiviral vector-based gene therapy to correct all mutations in CEP290.**
P083	CNS & sensory	T Taghian	*University of Massachusetts Medical School*	**Real-Time MR tracking of AAV gene therapy with β-gal activated MR probe**
P084	CNS & sensory	A Maddalena	*University of Bern*	**Comparison between CRISPR-mediated HITI and MITI for Opto-mGluR6 expression.**
P085	CNS & sensory	L K Finnegan	*Trinity College Dublin*	**Ablation of a prodegenerative gene confers histological and functional protection in a mouse model of retinal degeneration**
P086	CNS & sensory	S Gallorini	*Telethon Institute of Gene Therapy (HSR-TIGET)*	**Development of editing technologies for allele-specific silencing or precise correction of mutation hotspots affecting Alexander disease's patients**
P087	CNS & sensory	Y O Mukhamedshina	*Kazan Federal University*	**NG2-producing cells in the ventral horns with increasing distance from the spinal cord injury site**
P088	CNS & sensory	V Hernandez-Hernandez	*Institute of Child Health Great Ormond Street - UCL*	**Codon-optimisation for Bardet-Biedl Syndrome 1 (BBS1) and Bardet-Biedl Syndrome 10 (BBS10) genes for AAV constructs**
P089	CNS & sensory	S W Clark	*SwanBio Therapeutics*	**Intrathecal delivery of an AAV encoding human ABCD1 shows dose-responsive expression and activity in a mouse model of adrenomyeloneuropathy**
P090	CNS & sensory	V Vasireddy	*SwanBio Therapeutics*	**Three-Month Preclinical Safety Data of AAV9-hABCD1 following intrathecal delivery in Non-human Primates.**
P254	CNS & sensory	G Corcoran	*Sio Gene Therapies*	**Phase 1/2 Open-label Dose Evaluation Study of AXO-Lenti-PD Gene Therapy for Parkinson's Disease: Efficacy, Safety, and Tolerability Data up to 24 Months**

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GENE EDITING

No.	Category	Speaker	Speaker Affiliation	Poster Title
P092	Gene editing	A Hendel	*The Institute for Advanced Materials and Nanotechnology, The Mina and Everard Goodman Faculty of Life Sciences, Bar-Ilan University, Ramat-Gan 5290002, Israel*	**CRISPECTOR: an accurate tool for measuring CRISPR-Cas-induced translocation and adverse off-target activity**
P093	Gene editing	S Crippa	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Mesenchymal stromal cells improve the transplantation outcome of CRISPR-Cas9 gene-edited HSPCs.**
P094	Gene editing	B Liu	*Boston Children's Hospital*	**Development of a double shmiR lentivirus effectively targeting both BCL11A and ZNF410 for enhanced induction of fetal hemoglobin to treat β-hemoglobinopathies**
P095	Gene editing	K S Hinrichsmeyer	*Center for Drug Research, Ludwig-Maximilians-Universität München, Munich, Germany*	**Novel dual mRNA trans-splicing rAAV vectors for efficient reconstitution of split dCas9-VPR in neurons**
P096	Gene editing	L della Volpe	*San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), IRCCS San Raffaele Scientific Institute, Milan, Italy*	**Counteracting culture stress for efficient genetic engineering in hematopoietic stem and progenitor cells (HSPCs)**
P097	Gene editing	M WANG	*Department of Pediatrics, University of Minnesota, Minneapolis, Minnesota, 55455, USA*	**Multiplex base editing of NK cell to enhance cancer immunotherapy**
P098	Gene editing	L Benard	*Homology Medicines, Inc*	**HMI-103: An investigational gene editing vector for Phenylketonuria (PKU)**
P099	Gene editing	M C Castiello	*San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget), IRCSS San Raffaele Scientific Institute, Milan, Italy.*	**Targeted Genome Editing of Haematopoietic Stem Cells for Treating Recombination Activating Gene 1 (RAG1) Immunodeficiency**
P101	Gene editing	A Gutierrez-Guerrero	*CIRI; Inserm U1111*	**Baboon envelope pseudotyped "Nanoblades" loaded with Cas9/gRNAs complexes allow efficient genome editing in human T, B cells, HSCs and donor DNA knock-in provided by AAV-6 in HSCs**
P102	Gene editing	M Laurent	*Integrare research unit UMR_S951, Genethon, Inserm, Univ Evry, Université Paris-Saclay, 91000, Evry, France*	**Ex vivo editing of human hematopoietic stem cells for LAL-D therapy.**
P103	Gene editing	M Hussein	*Academic Medical Center*	**Novel CRISPR-Cas13d system to target highly conserved sequences of SARS-CoV-2 and other human coronaviruses**
P104	Gene editing	S Indik	*University of Veterinary Medicine, Vienna*	**Lentivector carrying Cas9 protein, guide RNA and transgene for simultaneous "hit-and-run" gene disruption and transgene delivery**

P105	Gene editing	A Shakirova	*RM Gorbacheva Research Institute of Pediatric Oncology, Hematology and Transplantation, Pavlov University*	**Innate immunity signaling inhibitors for stimulation of homology-directed repair at CCR5 locus during CCR5-Uco-TALEN-mediated genome editing in primary human hematopoietic stem cells.**
P106	Gene editing	R Rai	*UCL Institute of Child Health*	**Hematopoietic stem cell gene editing for the treatment of IL7R-SCID**
P108	Gene editing	M Dessy-Rodríguez	*CIEMAT/CIBERER*	**Modelling Congenital Dyserythropoietic Anemia Type II in Human Hematopoietic Stem and Progenitor Cells using Gene Editing**
P109	Gene editing	M. Pille	*University of Gent*	**CRISPR/Cas9-mediated gene editing for treatment of the Wiskott-Aldrich Syndrome**
P110	Gene editing	S Jalil	*University of Helsinki*	**Simultaneous high-efficiency base editing and reprogramming of patient fibroblasts**
P111	Gene editing	A A Shaimardanova	*Kazan Federal University*	**Assessment of functional activity of AAV9 encoding ARSA in the minipig model in vivo**
P112	Gene editing	D Hazelbaker*	*Vor Biopharma*	**In depth assessment of off-target editing by CRISPR/Cas9 in VOR33, an engineered hematopoietic stem cell transplant for the treatment of acute myeloid leukemia**
P113	Gene editing	J Klermund	*Institute for Transfusion Medicine and Gene Therapy, Medical Center – University of Freiburg, Freiburg, 79106, Germany*	**CAST-Seq discloses low frequency of chromosomal rearrangements in double-nickase based genome editing in primary human cells**
P114	Gene editing	M Palacios	*CIEMAT/CIBERER*	**Development of a gene editing therapeutic approach for the treatment of RPL5-deficient Diamond-Blackfan anemia patients**
P115	Gene editing	R Ferla	*Telethon Institute of Genetics and Medicine*	**AAV-mediated liver-directed homology-independent targeted integration provides stable therapeutic levels of transgene expression in newborn mice with a lysosomal storage disease.**
P116	Gene editing	I Petkovic	*EB House Austria, Research Program for Molecular Therapy of Genodermatoses, Department of Dermatology and Allergology, University Hospital of the Paracelsus Medical University Salzburg, 5020 Salzburg, Austria*	**COL17A1 editing in junctional epidermolysis bullosa keratinocytes via CRISPR/Cas9 induced homology-directed repair**

P117	Gene editing	C Brandas	*San Raffaele-Telethon Institute for Gene Therapy, IRCSS San Raffaele Scientific Institute, Milan, Italy.*	**Artificial Thymic Organoids as an in vitro platform to evaluate the T-cell differentiation potential of gene edited hematopoietic stem/progenitor cells**
P118	Gene editing	D Benati	*Department of Life Sciences, University of Modena and Reggio Emilia, Modena, Italy*	**CRISPR/Cas9 allele-specific design to inactivate a dominant-negative mutation in COL6A1 causing Ullrich muscular dystrophy**
P119	Gene editing	A Umbach	*Department CIBIO, University of Trento, via Sommarive 9, 38123 Trento, Italy*	**CRISPR-Cas9 mediated correction and generation of iPSC clones derived from a Cornelia de Lange Syndrome (CdLS) patient with a c.5483 G>A substitution in NIPBL**
P120	Gene editing	C Graham	*University College London*	**Development of a Gene Editing Strategy for Cystic Fibrosis**
P121	Gene editing	J C Buitrago	*Curexsys GmbH, Göttingen, 37079, Germany*	**Engineered human neonatal mesenchymal stromal cells expressing FGFb: assessment of immunophenotype, proliferation, differentiation and immunosuppressive effects in vitro.**
P122	Gene editing	I T Papademetriou	*MaxCyte*	**Preclinical optimization of gene editing for adoptive T cell therapy with high fidelity CRISPR/Cas9 ribonucleoproteins**
P123	Gene editing	J Hoersten	*Technical University Dresden*	**Surveying sequence specificity of tyrosine site-specific recombinases**
P124	Gene editing	M I Autio	*Genome Institute of Singapore*	**Computationally defined human genomic safe harbour loci validated in vitro for stable transgene expression**
P125	Gene editing	E V Bogoslovskaya	*Centre for Strategic Planning of FMBA of Russia*	**Evaluation of the efficiency of targeted transgene integration into the CCR5 gene locus using HDR and HITI.**
P126	Gene editing	K Singh	*Children's Hospital of Philadelphia*	**Efficient In Utero RNA Lipid Nanoparticle-mediated Gene Editing for Correction of Type 1 Tyrosinemia**
P127	Gene editing	E Segur-Bailach	*IDIBAPS*	**Generation of Glutaric Aciduria type-I cellular models and phenotype rescue by GCDH targeted gene edition.**
P128	Gene editing	V Noe	*Universidad de Barcelona*	**Exon skipping by Polypurine Reverse Hoogsteen hairpins**
P129	Gene editing	V Vavassori*	*San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan, Italy*	**Towards clinical translation of hematopoietic cell gene editing for treating Hyper-IgM Type 1**
P130	Gene editing	R Maldonado	*University of Helsinki*	**CRISPR/Cas9 correction of Finnish gyrate atrophy mutation in patient-derived iPSCs**

P133	Gene editing	F J Molina-Estévez	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**Modeling rare monogenic disorders as a translational tool: CRISPR/CAS9 editing of Pompe Mutations**
P134	Gene editing	B C Houghton	*UCL Institute of Child Health*	**Genome editing with TALEN, CRISPR Cas9 and Cas12a and AAV6 homology donor restores T cell function for XLP**
P135	Gene editing	I Ramos-Hernández	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**Optimizing genome editing approaches for safe transgene expression in monogenic disorders**
P137	Gene editing	Y Lin	*Department of Pharmacy and Center for NanoScience (CeNS), Ludwig-Maximilians-Universität, Munich, Germany.*	**Folate-targeted Cas9/sgRNA Ribonucleoprotein Delivery for Dual Immune Checkpoints Disruption in Colorectal Carcinoma**
P138	Gene editing	M Bulcaen	*KU Leuven*	**Base and prime editing as potential gene correction therapy for cystic fibrosis**
P139	Gene editing	N Ping	*University of Kyushu*	**Development of translational activation tool using designer PPR (Pentatricopeptide Repeat) proteins**
P140	Gene editing	A Hollingsworth	*GenEdit, Inc.*	**Polymer Nanoparticle Delivery of Firefly Luciferase mRNA Shows Distribution of Bioluminescence in the Brain**
P141	Gene editing	A Naseem	*UCL Institute of Child Health*	**Preclinical studies for a hematopoietic stem cell gene editing platform to treat Wiskott-Aldrich Syndrome.**
P142	Gene editing	E A Naumenko	*Kazan Federal University*	**Interaction of polyelectrolytes with DNA for gene delivery application**
P143	Gene editing	S Konstantinidou	*University of Pisa, Department of Biology*	**Improving delivery and genome editing efficiency of the CRISPR/Cas9 ribonucleoprotein applying a nanomedicine approach**
P144	Gene editing	B Akkaya	*The Department of Gene and Cell Therapy, Akdeniz University Faculty of Medicine, Antalya, 07058, Turkey*	**In vitro therapeutic evaluation of the lentiviral gene therapy vector encoding vasoactive intestinal peptide for retinal dystrophies**
P402	Gene editing	A Ghosh	*Division of RNA Biology & Cancer, German Cancer Research Center (DKFZ), 69120 Heidelberg, Germany / Division of Cancer Research, Department of Thoracic Surgery, Medical Center - University of Freiburg, Faculty of Medicine, University*	**Chimeric oligonucleotides containing guide RNA sequence and single-stranded DNA repair template effectively induce precision gene editing in mammalian cells.**

			of Freiburg, German Cancer Consortium (DKTK) - Partner Site Freiburg, 79106 Freiburg, Germany	

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GENE TARGETING

No.	Category	Speaker	Speaker Affiliation	Poster Title
P145	Gene targeting	K Olivieri	*Affinia Therapeutics*	**Combining distinct phenotypes of AAV capsid to simultaneously target and detarget specific tissues in mouse models**
P146	Gene targeting	A Kivitz	*Altoona Center for Clinical Research*	**A first-in-human, phase 1, open-label trial of FX201, an intra-articular, helper-dependent adenoviral gene therapy for osteoarthritis – preliminary evaluation of clinical activity in 8 patients from the mid-dose cohort of a single ascending dose study design**
P147	Gene targeting	E Zinn	*Grousbeck Gene Therapy Center, Schepens Eye Research Institute, Massachusetts Eye and Ear, Boston, MA, USA*	**Combinatorial AAV Capsid Library Enables Multidimensional Study of Vector Biology**
P148	Gene targeting	A GARANTO	*Department of Pediatrics, Radboud University Medical Center*	**Splicing modulation therapy for a variety of ABCA4 mutations underlying Stargardt disease**
P149	Gene targeting	M Lisjak	*ICGEB*	**Non-integrative and integrative gene-based strategies for the treatment of Citrullinemia type I mouse model**
P150	Gene targeting	M Colantuoni	*San Raffaele Telethon Institute for Gene Therapy (SR TIGET), IRCCS San Raffaele Scientific Institute, Milan, Italy*	**Gene therapy for IL-1-mediated systemic autoinflammatory diseases**
P151	Gene targeting	M GE Rommel	*Paul-Ehrlich-Institute*	**Targeting Therapeutic Proteins to Alpha Granules for Platelet-mediated Transport and Delivery**
P152	Gene targeting	R Ferla	*Telethon Institute of Genetics and Medicine*	**Towards a clinical trial of gene therapy to target retinitis pigmentosa associated with Usher syndrome type IB**
P153	Gene targeting	Y Sharma	*Homology Medicines, Inc.*	**Gene therapy-mAb platform targets Complement Protein 5 using AAVHSCs**
P154	Gene targeting	A PJ Giese	*Sensorion*	**In vivo translation of adeno-associated vectors for the treatment of inner ear disorders.**
P155	Gene targeting	W Schäfer	*University Medical Center Hamburg-Eppendorf*	**Enhancing cell specificity of AAV gene therapy vectors by using membrane-protein specific nanobodies**

P156	Gene targeting	M A Vigovskii	*Institute for Regenerative Medicine, Lomonosov Moscow State University, Moscow, Russia*	**Single cell RNA-seq analysis of multipotent mesenchymal stromal cells revealed polarly expressed genes during myofibroblast and adipogenic differentiation.**
P157	Gene targeting	L Santi	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Dissecting bone remodelling mechanisms and hematopoietic stem cell gene therapy impact in Mucopolysaccharidosis type I Hurler bone defects**
P158	Gene targeting	P Puig-Serra	*Centro Nacional de Investigaciones Oncológicas*	**Transient and lentiviral delivery of CRISPR/Cas13 system to knockdown oncogenes**
P159	Gene targeting	M Jelicic	*Medical Systems Biology, Medical Faculty, Technical University Dresden*	**Characterisation of novel naturally occurring site-specific recombinase systems**
P160	Gene targeting	P S Loh	*Department of Microbiology & Immunology, Yong Loo Lin School of Medicine, National University of Singapore*	**Design, Generation, and Investigation of Novel Dumbbell-Shaped DNA Minimal Vectors for Suicide Gene Therapy**
P161	Gene targeting	S Valiuska	*University of Barcelona*	**Gene silencing of c-MYC and K-RAS oncogenes by targeting G-quadruplexes using Polypurine Reverse Hoogsteen Hairpins**
P162	Gene targeting	S V Shtykalova	*D.O.Ott Research Institute of Obstetrics, Gynecology and Reproductology, Saint-Petersburg, Russian Federation*	**Peptide-based carriers with anionic coating as a delivery system for gene therapy of uterine leiomyoma.**
P163	Gene targeting	K Piekarowicz	*University of Wroclaw*	**The development of Hutchinson-Gilford progeria syndrome gene therapy using RNA interference.**
P164	Gene targeting	A J Forbes	*Gene Regulation, MeiraGTx, New York, NY 10016, USA*	**Novel riboswitches regulate AAV delivered transgene expression in mammals via small molecule inducers**
P165	Gene targeting	M Fan	*Amsterdam UMC*	**Extinction of all infectious HIV in cell culture by the CRISPR-Cas12b system**
P166	Gene targeting	I Pérez de Castro	*Instituto de Salud Carlos III*	**Using CRISPR/Cas9 technology for the specific elimination of dominant mutations associated with rare diseases**
P167	Gene targeting	C Mason	*AVROBIO, Inc.*	**The GuardOne clinical trial: a first in-human, open-label, multinational phase 1/2 study of AVR-RD-02 ex vivo lentiviral vector, autologous gene therapy for Gaucher disease**
P168	Gene targeting	E Aubets	*University of Barcelona*	**RNA-PolyPurine Reverse Hoogsteen Hairpins as tool for gene silencing**
P169	Gene targeting	I Gómez-Aguado	*Pharmacokinetic, Nanotechnology and Gene Therapy Group (PharmaNanoGene), Faculty of Pharmacy, Centro de investigación Lascaray ikergunea, University of the Basque Country UPV/EHU, Paseo de la Universidad 7,*	**Lipid based vectors containing mRNA for corneal inflammation treatment**

			01006 Vitoria-Gasteiz, Spain; Bioaraba, PharmaNanoGene, Vitoria-Gasteiz, Spain	
P170	Gene targeting	L Medaer	*KU Leuven*	**Development of isogenic myoblast-based models to study cystinosis myopathy**
P171	Gene targeting	S Blondel	*Yposkesi*	**Targeting cost-effective large-scale manufacturing for r-AAV vectors by transient transfection using Pall's Allegro™ STR bioreactor**

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HEMATOPOIETIC & BLOOD DISORDERS

No.	Category	Speaker	Speaker Affiliation	Poster Title
P172	Hematopoietic & blood disorders	I Moscatelli	*Department of Molecular Medicine and Gene Therapy, Lund Strategic Center for Stem Cell Biology, Lund, Sweden*	**Preclinical efficacy and safety of lentiviral (LV)-based gene therapy in IMO support clinical trial initiation**
P173	Hematopoietic & blood disorders	V Capo	*San Raffaele-Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, 20132, Italy*	**Efficacy assessment of lentiviral vector gene therapy in the immune dysregulated hypomorphic Rag1 mice**
P174	Hematopoietic & blood disorders	L Basso-Ricci	*San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), San Raffaele Scientific Institute, Milan, Italy*	**Kinetics and composition of haematopoietic stem/progenitors mobilized cells upon G-CSF and Plerixafor administration in transplant donor or patients undergoing autologous gene therapy.**
P175	Hematopoietic & blood disorders	C Canepari	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Improving the efficacy of liver directed lentiviral gene therapy for hemophilia**
P176	Hematopoietic & blood disorders	L Bucciarelli	*Pediatric Hematology and Oncology Center, Woman's and Child Health Department, University of Padova, Padova, Italy*	**Development of an ex vivo Gene Therapy for Infantile GM1-Gangliosidosis**
P177	Hematopoietic & blood disorders	A Mortellaro	*San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute*	**Lentiviral-mediated gene therapy for the treatment of adenosine deaminase 2 deficiency**
P178	Hematopoietic & blood disorders	Y Gimenez	*CIEMAT/CIBERER*	**Preclinical studies for lentiviral-mediated gene therapy of RPS19-deficient Diamond-Blackfan anemia**
P179	Hematopoietic & blood disorders	R Hervas-Salcedo	*CIEMAT-CIBERER/IIS-FJD*	**Mesenchymal stromal cells stably expressing CXCR4 and IL10 as therapy for acute graft versus host disease.**
P180	Hematopoietic & blood disorders	A Al-Mohannadi	*Research Department, Sidra Medicine, PO Box 26999, Doha, Qatar*	**Targeting CD34+ Hematopoietic Stem cells: a potential promise for Hemophilia A**

P181	Hematopoietic & blood disorders	A Calabria	*San Raffaele Telethon Insitute for Gene Therapy (HSR-TIGET)*	**Hematopoietic reconstitution and lineage commitment in HSC GT patients are influenced by the disease background**
P182	Hematopoietic & blood disorders	B Yates	*BioMarin Pharmaceutical*	**Investigating Mechanisms of Variability of AAV5-hFVIII-SQ Expression in Mice**
P183	Hematopoietic & blood disorders	M Fernández-García	*CIEMAT/CIBERER/IIS-FJD*	**Fanconi anemia mesenchymal stromal cells have reduced differentiation capacity and impaired HSC maintenance**
P184	Hematopoietic & blood disorders	E Tsempera	*Laboratory of Cell and Gene Therapy, Centre for Basic Research, Biomedical Research Foundation of the Academy of Athens (BRFAA), Athens, Greece*	**A novel γ-globin lentiviral vector pseudotyped with the HF glycoproteins restores the thalassemic maturation bottleneck and displays a safe integration pattern**
P185	Hematopoietic & blood disorders	P Tajer	*Leiden University Medical Center*	**Ex vivo expansion of Human Hematopoietic Stem Cells for Gene and Cell Based Therapies**
P186	Hematopoietic & blood disorders	P Quaranta	*San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan, Italy*	**Role of peripheral blood circulating haematopoietic stem/progenitor cells during physiological haematopoietic maturation and after gene therapy**

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IMMUNOTHERAPY & CAR T CELLS

No.	Category	Speaker	Speaker Affiliation	Poster Title
P187	Immunotherapy & CAR T cells	F Manfredi	*Vita-Salute San Raffaele University*	**Patient-derived, exhausted tumor-specific T cells can be manipulated to establish a T-cell receptors library for adoptive T cell therapy**
P188	Immunotherapy & CAR T cells	P Justicia-Lirio	*LentiStem Biotech*	**Development of physiological and inducible 4th generation CAR (iTRUCK)-T cells using the Lent-On-Plus platform**
P189	Immunotherapy & CAR T cells	K Pavlovic	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**Improvement of "off-the-shelf" allogeneic CAR-T cells**
P190	Immunotherapy & CAR T cells	M Belver	*Institute of Biology and Molecular Genetics (IBGM), University of Valladolid (UVa)-CSIC, Valladolid, Spain.*	**Efficient AAV/CRISPR-based genetic engineering strategy for the production of allogeneic NKG2D CAR T cells in a single step production of allogeneic NKG2D CAR T cells in a single step process**
P191	Immunotherapy & CAR T cells	A Miodek	*Ixaka, Villejuif, France*	**Engineering and functional characterization of anti-PSMAxCD3 bispecific aptamers**
P192	Immunotherapy & CAR T cells	P Gee	*MaxCyte, Inc.*	**Non-Viral Engineering of Human iPS Cells to Manufacture Rejuvenated TCR T Cells for Cancer Immunotherapy**

P194	Immunotherapy & CAR T cells	A Galy	*Genethon*	**Human syncytins can pseudotype lentiviral vectors, enabling gene transfer into B cells**
P195	Immunotherapy & CAR T cells	S Feola	*University of Helsinki*	**A novel immunopeptidomic-based pipeline for the generation of personalized oncolytic cancer vaccines**
P196	Immunotherapy & CAR T cells	C Duthoit	*Flash Therapeutics*	**Efficient and safe delivery of multiple mRNA using non-integrative bacteriophage-chimeric retrovirus-like particles for cell therapy and vaccination perspectives.**
P197	Immunotherapy & CAR T cells	P Barbao	*IDIBAPS*	**Characterization of CAR-mediated T cell dysfunction in mice with solid tumors**
P198	Immunotherapy & CAR T cells	N Ho	*Paul-Ehrlich-Institut*	**In vivo CAR T cell generation in humanized NSG-SGM3 mice**
P199	Immunotherapy & CAR T cells	B Gentner	*San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)*	**TEM-GBM: an open-label, Phase I/IIa dose-escalation study evaluating the safety and efficacy of genetically modified Tie-2 expressing monocytes to deliver IFN-a within glioblastoma tumor microenvironment**
P200	Immunotherapy & CAR T cells	B Fehse	*UMC Hamburg-Eppendorf*	**Real-world experience with axicabtagene ciloleucel in aggressive B-cell lymphoma confirms correlation of in-vivo expansion with treatment outcome**
P201	Immunotherapy & CAR T cells	J Gertner-Dardenne	*Sangamo Therapeutics*	**IL23R-CAR-Tregs - a novel approach to treat Crohn's disease patients**
P202	Immunotherapy & CAR T cells	J L McGovern	*Quell Therapeutics*	**Engineering Regulatory T cells with constitutive expression of FOXP3 as a phenotype-lock enhances efficacy and safety of Treg cell therapies**
P203	Immunotherapy & CAR T cells	B Perucha	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**CAR T cell derived exosomes: structural and functional characterization.**
P204	Immunotherapy & CAR T cells	E Ruggiero	*Experimental Hematology Unit, Division of Immunology, Transplantation and Infectious Diseases, San Raffaele Scientific Institute, Milan, Italy*	**Exploiting genome editing and WT1-specific T cell receptors to redirect T lymphocytes against acute myeloid leukemia**
P205	Immunotherapy & CAR T cells	A Prodeus	*Intellia Therapeutics*	**Lipid nanoparticles (LNPs) as a superior CRISPR/Cas9 delivery modality for highly efficient multiplex gene editing of T cells for adoptive cell therapy**
P206	Immunotherapy & CAR T cells	D Blount	*Oxford BioMedica*	**Pre-clinical development of CAR-T Cells directed against the tumour antigen 5T4 for the treatment of Acute Myeloid Leukaemia**
P207	Immunotherapy & CAR T cells	A Szoor	*University of Debrecen*	**Cytolytic activity of CAR T cells and maintenance of their CD4+ fraction is critical for optimal antitumor activity in preclincial solid tumor models**
P208	Immunotherapy & CAR T cells	C Aparicio	*Institute of Biology and Molecular Genetics (IBGM), University of Valladolid (UVa)-CSIC, Valladolid, Spain.*	**Allogeneic NKG2D-CAR T cells as universal anticancer treatment: new manufacturing strategy based on CRISPR/Cas9**

P209	Immunotherapy & CAR T cells	M Fusciello	*TRIMM, Translational Immunology Research Program, University of Helsinki, Finland*	**Novel personalized cancer vaccine platform based on Bacillus Calmette-Guèrin**
P210	Immunotherapy & CAR T cells	A Miodek	*Ixaka*	**Functional characterization of CD3-specific DNA aptamers**
P211	Immunotherapy & CAR T cells	C F Pires	*Wallenberg Centre for Molecular Medicine, Lund University, Lund, 221 84, Sweden.*	**Single-cell RNA-seq informs efficient reprogramming of human somatic cells to cross-presenting dendritic cells**
P212	Immunotherapy & CAR T cells	A Stamopoulou	*Institute of Experimental Hematology, Hannover Medical School, Hannover, 30625, Germany*	**Chimeric Antigen Receptor (CAR)-T cells targeting EpCAM for the treatment of pediatric Germ Cell Tumors (pGCTs)**
P214	Immunotherapy & CAR T cells	R Pacherie	*IXAKA*	**Targeted Nano-particles (TNP) technology as a universal and versatile platform for gene therapy.**
P215	Immunotherapy & CAR T cells	M Fusciello	*University of Helsinki*	**A personalized anti-cancer vaccine for melanoma based on an approved vaccine against measles, mumps, and rubella**
P216	Immunotherapy & CAR T cells	N Maldonado-Pérez	*Fundacion Publica Andaluza Progreso y Salud/Genyo*	**Feasibility of generating universal and physiological CAR T cells for the treatment of type B malignances.**
P217	Immunotherapy & CAR T cells	B Drees	*Miltenyi Biotec*	**Manufacturing of tightly controllable Adapter CAR T cells using the CliniMACS Prodigy® platform**
P218	Immunotherapy & CAR T cells	A Westhaus	*Children's Medical Research Institute, University of Sydney*	**Selecting AAV capsids for optimised homology-dependent repair in T cells.**
P219	Immunotherapy & CAR T cells	L García-García	*Fundación de Investigación Biomédica, Hospital Infantil Universitario Niño Jesús, Madrid, 28009, Spain*	**Combination of nanomedicine and immunotherapy for the treatment of Neuroblastoma**
P220	Immunotherapy & CAR T cells	O A Rodrigues	*University of São Paulo*	**Induction of immunogenic cell death in canine melanoma model in response to combined p14ARF and interferon-beta gene transfer.**
P221	Immunotherapy & CAR T cells	M Lysandrou	*University of Patras Medical School*	**Decitabine induces IDO1 expression on human CD4+ T cells**
P222	Immunotherapy & CAR T cells	L Hidalgo	*Cellular Biotechnology Unit, Instituto de Salud Carlos III*	**Antitumor responses of anti-FITC CAR-T cells targeting B7-H3+ sarcoma tumor cells.**
P224	Immunotherapy & CAR T cells	E M McErlean	*Queen's University Belfast*	**Peptide-based nanoparticles for ex vivo genetic modification of natural killer cells**
P225	Immunotherapy & CAR T cells	M Cortijo-Gutiérrez	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**Creation of next generation CAR-T by Repurposing endogenous immune pathways**

P226	Immunotherapy & CAR T cells	S Russo	*University of Helsinki*	**Exploiting epigenetic therapy to improve viral-based platforms for cancer immunotherapy.**
P227	Immunotherapy & CAR T cells	C Johnson	*Bio-Techne, 3F Gill Street Woburn, MA 01801, USA*	**Expansion of natural killer (NK) cells using an FBS-free and feeder cell-free culture protocol**
P228	Immunotherapy & CAR T cells	I Y Filin	*Institute of Fundamental Medicine and Biology, Kazan Federal University, Kazan, Russia*	**Analysis if CD markers of immune cells after interaction with melanoma cells membrane vesicles**

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INFECTIOUS DISEASES

No.	Category	Speaker	Speaker Affiliation	Poster Title
P229	Infectious diseases	U Bhatt	*Grousbeck Gene Therapy Center, Schepens Eye Research Institute, Mass Eye and Ear, Boston, MA*	**AAVCOVID, a single dose AAV-based vaccine, elicits potent and broad SARS-CoV-2 neutralizing response**
P230	Infectious diseases	G Magro	*Università degli Studi di Padova*	**Development of a CRISPR/Cas9 vector driven by a cell-activation-inducible promoter for HIV-1 gene editing**
P231	Infectious diseases	E Vamva	*University of Washington*	**Primary B cell restricted eCD4-Ig expression through lentiviral vector delivery supports HIV-1 neutralisation**
P232	Infectious diseases	E Herrera Carrillo	*Laboratory of Experimental Virology, Department of Medical Microbiology, Amsterdam UMC, Academic Medical Center, University of Amsterdam, Amsterdam, the Netherlands*	**Novel vector systems towards a cure for HIV/AIDS**
P233	Infectious diseases	F Erendor	*The Department of Gene and Cell Therapy, Akdeniz University Faculty of Medicine, Antalya, 07058, Turkey*	**Construction of a lentiviral vector-carrying SARS-CoV-2 spike gene for COVID-19**
P234	Infectious diseases	A O Rozhin	*Kazan Federal Universiry*	**Combained antibacterial effect of polyethyleneimine and halloysite nanotubes**
P235	Infectious diseases	E O Sahin	*The Department of Gene and Cell Therapy, Akdeniz University, Faculty of Medicine, Antalya, 07058, Turkey*	**Adenoviral vector design and construction for SARS-CoV-2 vaccine development**
P236	Infectious diseases	A O Rozhin	*Kazan Federal University*	**Investigation of the antibacterial activity of a composite based on halloysite nanotubes and silver nanoparticles**

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LIVER & METABOLIC				
No.	**Category**	**Speaker**	**Speaker Affiliation**	**Poster Title**
P237	Liver & metabolic	T Tsunogai	*Department of Pediatrics, Jikei University School of Medicine*	**Hematopoietic stem cell gene therapy corrects lysosomal storage in CNS in murine model of GM1-gangliosidosis**
P238	Liver & metabolic	M Milani	*San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)*	**Lentiviral vector mediated in vivo gene transfer into liver organoid forming cells in mice and non-human primates**
P240	Liver & metabolic	Y M Lee	*Glycogen Storage Disease Program Department of Pediatrics University of Connecticut School of Medicine*	**Sustained correction of Glycogen Storage Disease Type Ib mice by gene therapy**
P241	Liver & metabolic	A Molinos-Vicente	*Biomedical Innovation Unit, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER), Madrid, Spain.*	**Preclinical development of an in vivo lentiviral gene therapy for the treatment of Primary Hyperoxaluria Type 1**
P242	Liver & metabolic	L Jauze	*GENETHON*	**A novel therapeutic strategy for skeletal disorders: proof-of-concept of gene therapy for X-linked hypophosphatemia**
P243	Liver & metabolic	P O Ilyinskii	*Selecta Biosciences*	**Dose finding study of AAV-LSP-MMUT in a mouse model of MMA and efficient suppression of anti-capsid antibody responses by single and multiple administrations of ImmTOR nanoparticles**
P244	Liver & metabolic	L Touramanidou	*Genetics and Genomics Medicine Group, Great Ormond Street Institute of Child Health, University College London, London, UK*	**In vivo lentiviral gene therapy for argininosuccinic aciduria**
P245	Liver & metabolic	N D Weber	*Vivet Therapeutics, S.L., Pamplona, 31008, Spain*	**Re-Administration of AAV expressing MDR3 (VTX-803) treats progressive familial intrahepatic cholestasis type 3 (PFIC3) in juvenile Abcb4−/− mice when co-administered with ImmTOR**
P246	Liver & metabolic	A Mateu-Bosch	*IDIBAPS*	**GCDH gene therapy shows efficacy in the glutaric aciduria type I mouse model.**
P247	Liver & metabolic	J Martínez-García	*CIMA Universidad de Navarra*	**A minimal BSEP promoter allows bile acid-driven physiological regulation of transgene expression from an AAV vector**
P248	Liver & metabolic	A Bazo	*Division of Gene Therapy and Regulation of Gene Expression, Cima Universidad de Navarra, Pamplona, Spain.*	**rAAV-mediated gene therapy in combination with short-term nitrogen-scavenger treatment corrects biochemical and behavioral abnormalities and increases lifespan in infant Citrullinemia Type 1 (CTLN-1) mice**

P249	Liver & metabolic	S Lumbreras	*Universidad de Navarra*	**Gene supplementation of CYP27A1 in the liver restores bile acid metabolism in a mouse model of Cerebrotendinous Xanthomatosis**
P250	Liver & metabolic	A C Cozmescu	*Gene Therapy for Metabolic Diseases, NIHR Great Ormond Street Hospital Biomedical Research Centre, University College London, London, WC1N 1EH, United Kingdom*	**Lentiviral gene therapy for arthrogryposis-renal dysfunction-cholestasis (ARC) syndrome**
P251	Liver & metabolic	I Stafeev	*National Medical Research Centre for Cardiology*	**Baculoviral transduction of 3T3-L1 adipocytes with CRISPR/dCas9 system activating UCP-1 expression increases glucose uptake**
P252	Liver & metabolic	E Mamontova	*Lomonosov Moscow State University*	**Activation of UCP-1 expression in 3T3-L1 cells via CRISPR/dCas9 system leads to decrease of cytochrome c oxidase and superoxide dismutase genes expression**
P253	Liver & metabolic	Q Xiong	*LogicBio Therapeutics*	**A novel endonuclease-free genome editing technology to edit hepatocytes in vivo led to a full molecular liver transplant and cured mice in preclinical models of Tyrosinemia Type 1**
P255	Liver & metabolic	N Boulos	*REGENXBIO*	**Evaluation of a CSF biomarker for gene therapy that discriminates neuronopathic and non-neuronopathic forms of MPS II**

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MANUFACTURING

No.	Category	Speaker	Speaker Affiliation	Poster Title
P256	Manufacturing	A Dane	*Freeline, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX UK*	**RNAScope™ analysis of non-human primate liver after AAVS3 delivery shows widespread liver transduction and periportal transgene expression**
P257	Manufacturing	N Faulkner	*National Heart and Lung Institute, Imperial College London*	**Reducing anti-vector humoral immunity through modulation of surface glycoprotein density**
P258	Manufacturing	A Magnusson	*Cytiva*	**Development of a scalable adeno-associated virus production process by transient transfection in suspension cells**
P259	Manufacturing	L I Schwarze	*Dept. of Stem Cell Transplantation, Research Dept. Cell and Gene Therapy, University Medical Center Hamburg-Eppendorf*	**GMP-compatible, automated production of CCR5-edited CD4+-T cells for the treatment of HIV-positive patients**
P260	Manufacturing	A Schulze	*Freeline, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX UK*	**A second-generation two-plasmid packaging system for manufacturing of AAV vectors further improves quality and yield**

P261	Manufacturing	L Galibert	*Kuopio Center for Gene and Cell Therapy*	**Functional Roles of the Novel Membrane-Associated AAV Protein MAAP**
P263	Manufacturing	H Laux	*CSL Behring Innovation GmbH*	**Comparison of Lentiviral Vector Production from two Stable Packaging Cell Lines**
P264	Manufacturing	D Klatt	*Gene Therapy Program, Dana Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School*	**Genetic engineering of a virus producer cell line to generate high-titer and high-quality BaEVRless-pseudotyped alpha-retroviral particles**
P265	Manufacturing	N Hazi	*Pall Life Sciences*	**Application of Aber's FUTURA♦ Biomass Probes to Inform Transfection and Cell Lysis in iCELLis® Bioreactor-based AAV Manufacturing**
P266	Manufacturing	K Hein	*Cevec Pharmaceuticals GmbH, Cologne*	**High titer rAAV production in bioreactor using ELEVECTA® stable producer cell lines**
P268	Manufacturing	W Lee	*LogicBio Therapeutics*	**Development of an Anion Exchange Chromatography Method to Assess Percent Full Capsids for Chimeric Capsid AAV-LK03**
P269	Manufacturing	A Hagner-McWhirter	*Cytiva*	**Development of a scalable adeno associated virus purification process and an SPR titer assay**
P270	Manufacturing	A Soula	*Cell Therapy Catapult*	**AAV production using a closed, semi-automated hollow fibre bioreactor**
P271	Manufacturing	S Elavazhagan	*Orchard Therapeutics Ltd*	**Optimized Lentiviral Transduction Process for ex vivo CD34+ Hematopoietic Stem Cell Gene Therapy Drug Product Manufacture**
P272	Manufacturing	J T ISSENHUTH	*Polyplus Transfection*	**Combining highest rAAV manufacturing performance with highest quality standards to support CGT industry**
P273	Manufacturing	B Handyside	*BioMarin Pharmaceutical*	**Similar long-term durability of expression in mice dosed with adeno-associated virus (AAV) vector produced in HEK293 versus Sf cell lines**
P274	Manufacturing	M Schofield	*Pall Life Sciences*	**Separation of full and empty AAV capsids using membrane chromatography**
P275	Manufacturing	J S Ketz	*Andelyn Biosciences*	**A DoE Approach to optimize AAV production and purification using FectoVir®-AAV.**
P276	Manufacturing	A Arrasate	*VIVEbiotech*	**Isolation and characterization of cGMP monoclonal HEK293T master cell bank to manufacture lentiviral vector in adherence-based bioreactors under serum-free conditions**
P277	Manufacturing	A Rose	*Pall Life Sciences*	**Nanofiltration of AAV Cell Culture Media**
P278	Manufacturing	W H Reuter	*LogicBio Therapeutics*	**Modified plasmid and transfection optimization in suspension HEK293 cells lead to scalable high-yield process for AAV manufacturing**

P279	Manufacturing	C Lopez	*VIVEbiotech*	**Pushing the right buttons: genetic silencing in producing cell lines to boost lentiviral vector production led to metabolic disfunction counteracting vector requirements**
P280	Manufacturing	M Neri	*AGCBiologics*	**Meeting Market Needs with Scalable AAV Manufacturing**
P281	Manufacturing	J Zanker	*Charité - Universitätsmedizin Berlin, Germany*	**Superior precision and reliability of multidimensional ddPCR for accurate analysis of rAAV vectors**
P282	Manufacturing	T Dempton	*IXAKA*	**Ixaka versatile platform for the bioproduction and characterisation of lentiviral vectors: from R&D quality up to pilot-grade batches**
P283	Manufacturing	C Lachica	*Sidra Medicine, Doha, Qatar*	**Stability and proliferative potential of hematopoietic stem cells is maintained over time after thawing in albumin solution.**
P284	Manufacturing	M Fertin	*VIVEbiotech*	**Lentiviral CAR-T backbone promotor configuration determine vector production yield and vector functionality by modulating viral genome production and packaging yields**
P285	Manufacturing	M Penaud-Budloo	*INSERM UMR 1089, University of Nantes, CHU of Nantes*	**High added value of DoE and Ambr®15 automated bioreactor for determining critical parameters of AAV vector production in mammalian and insect suspension cells**
P286	Manufacturing	B Hudjetz	*Cevec Pharmaceuticals GmbH, Cologne*	**Development of a HEK293-based fully stable, helper virus-free ELEVECTA production system for rAAV**
P287	Manufacturing	R GAYON	*Flash Therapeutics*	**A same lentiviral vector manufacturing process for both research and clinical application**
P289	Manufacturing	G De Carluccio	*Università degli Studi di Napoli Federico II*	**A synthetic biology approach to large scale AAV production**
P290	Manufacturing	N Y GO	*Toolgen Inc.*	**Enhancing transduction efficiency of Adeno-Associated Virus 9 by cell line gene engineering for gene therapy potency assay**
P291	Manufacturing	N Kraemer	*Xell AG*	**Form follows function – or vice versa? The effect of plasmid quality and transfection reagent on transfection, cytotoxicity and AAV production in HEK cells**
P292	Manufacturing	N Hazi	*Pall Life Sciences*	**Efficient Lentiviral Vector Production in a Chemically Defined, Blood-Free and Serum-Free Medium, Scalable to the iCELLis® Technology**
P293	Manufacturing	D L Martins	*Baxalta Innovations GmbH, a part of Takeda*	**Robotics & Miniaturization enable fast serotype adaptation of AAV DSP-Platform**
P294	Manufacturing	T R Thomas Robert	*Univercells Technologies*	**Integrated & continuous processing: a proven solution to tackle gene therapy manufacturing challenges**
P295	Manufacturing	K Bayne	*Pall Life Sciences*	**Scale Up of a Lentiviral Production Process from the iCELLis® Nano Bioreactor to the iCELLis 500 + Bioreactor**
P296	Manufacturing	E Guzniczak	*RoslinCT, Nine Edinburgh BioQuarter, 9 Little France Road, Edinburgh*	**Development of manufacturing system for GMP-grade iPSC lines**

P297	Manufacturing	M Malm	*Royal Institute of Technology*	**Improving targeting and yield of AAV by capsid and cell engineering**
P298	Manufacturing	D Holzinger	*PROGEN*	**AAV VP1, VP2 and VP3 protein standards – Value of reliable standards for AAV vector manufacturing**
P300	Manufacturing	J Wright	*Oxford BioMedica*	**Major splice donor-mutated HIV-1 lentiviral vectors (LVs) and enhancement of their production by co-expression of LV RNA-targeted U1 snRNA.**
P301	Manufacturing	M Janc	*Department of Biotechnology and Systems Biology, National institute of Biology, Vecna pot 111, Ljubljana, 1000, Slovenia*	**In depth analysis of AAV-containing fractions extracted from CsCl ultracentrifugation gradient**
P302	Manufacturing	E Fong	*Merck Millipore*	**Development and scale-up of a transient AAV production process using a suspension-adapted HEK293 clone**
P303	Manufacturing	G Vallanti	*AGC Biologics*	**LVV Production At-Scale: From Cell Factory™ Systems to iCELLis® 500 Bioreactor**
P304	Manufacturing	Y ERIC Yao	*University of Iowa*	**Upstream Process Optimization of an Oncolytic Vaccinia Virus Production**

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MUSCLE DISEASES

No.	Category	Speaker	Speaker Affiliation	Poster Title
P305	Muscle diseases	A Kagiava	*Neuroscience Department, The Cyprus Institute of Neurology and Genetics and Cyprus School of Molecular Medicine*	**AAV9 gene replacement therapy in two mutant mouse models of CMT1X demyelinating neuropathy**
P306	Muscle diseases	P Sellier	*Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*	**CRISPR/Cas9-mediated glycogen synthase silencing as effective substrate reduction therapy in glycogen storage diseases mouse models.**
P308	Muscle diseases	O Danos	*REGENXBIO Inc*	**A Novel AAV8-Based Gene Therapy for Duchenne Muscular Dystrophy: Preclinical Studies in the Mdx Mouse**
P309	Muscle diseases	C W Chen	*Astellas Gene Therapies*	**Large animal safety studies demonstrate no drug-related toxicity following high doses of AT132**
P311	Muscle diseases	V Cernisova	*Royal Holloway, University of London*	**Improvement of skeletal muscle function in the Dba2.mdx mouse following administration of AAV-microdystrophin**
P312	Muscle diseases	C Daoud	*Université Paris-Saclay, Univ Evry, Inserm, Généthon, Integrare research unit UMR_S951, 91000, Evry-Courcouronnes, France*	**Characterization of a new Duchenne Muscular Dystrophy rat model with an out-of-frame deletion of exon 45**

P313	Muscle diseases	C Vaubourg	*Université Paris-Saclay, Univ Evry, Inserm, Généthon, Integrare research unit UMR_S951, 91000, Evry-Courcouronnes, France*	**Effects of Smad7 gene transfer in a DMD mouse model**
P314	Muscle diseases	M Geoffroy	*Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*	**Dose escalation preclinical study with FKRP gene therapy and benefits on dysregulated pathways**
P315	Muscle diseases	A Aguilar-González	*GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia*	**Generation of Pompe disease cellular models to analyze gene therapy aproaches using optimized GAA**
P316	Muscle diseases	S Guiraud	*Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*	**Development of a utrophin modulation CRISPR-Cas9 strategy for Duchenne Muscular Dystrophy**

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NON CODING RNA

No.	Category	Speaker	Speaker Affiliation	Poster Title
P317	Non coding RNA	S Alsing	*Aarhus University, Department of Biomedicine*	**Pol II-driven Dicer-independent shRNAs provide efficacy and improved safety for RNAi-based gene therapy**
P318	Non coding RNA	S Carrella	*Telethon Institute of Genetics and Medicine (TIGEM)*	**AAV-Sponge-mediated modulation of miR-181a/b as a gene-independent therapeutic approach for inherited retinal diseases (IRDs)**
P319	Non coding RNA	S Hamza	*Kazan Federal University*	**Trapping miR-223 leads to overexpression of NLRP3 in colorectal cancer**
P320	Non coding RNA	M Volpe	*TIGEM*	**AAV-mediated inhibition of miR-181a/b as gene-independent therapeutic tool for mitochondrial diseases**

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ONCOLYTIC VIRUSES

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P321	Oncolytic viruses	M Fusciello	*University of Helsinki*	**Characterization of a novel OX40 ligand and CD40 ligand-expressing oncolytic adenovirus used in the PeptiCRAd cancer vaccine platform**
P322	Oncolytic viruses	G Herrador-Cañete	*CIMA Universidad de Navarra*	**Expression of galectin-3 inhibitors from a self-replicating RNA vector can inhibit the growth of pediatric osteosarcoma**

P323	Oncolytic viruses	R Nilson	*Ulm University*	**Hexon modification of HAdV-5 vectors enables efficient transduction of human multipotent mesenchymal stromal cells**
P324	Oncolytic viruses	E Sallard	*Witten/Herdecke University*	**Development of oncolytic and gene therapy vectors based on Adenovirus serotype 4 as an alternative to Adenovirus serotype 5**
P325	Oncolytic viruses	P Cordelier	*Inserm*	**Directed evolution generates novel oncolytic H-1 parvoviruses with improved therapeutic efficacy in virus-resistant pancreatic cancer cells**
P326	Oncolytic viruses	L Kuryk	*National Institute of Public Health NIH – National Research Institute*	**The combinatory therapy of oncolytic adenovirus armed with co-stimulatory molecules and anti-PD1 as a promising malignant mesothelioma treatment strategy**
P327	Oncolytic viruses	A Morales-Molina	*Cellular Biotechnology Unit, Instituto de Salud Carlos III*	**A novel oncolytic virus (ISC301) effectively inhibits tumor growth based on the intratumoral activation of the immune system**
P328	Oncolytic viruses	M Garofalo	*University of Padova*	**Turning melanoma cold tumors into hot by enhancing T-cell infiltration**
P329	Oncolytic viruses	L Krutzke	*Ulm University*	**Chorioallantoic Membrane tumour model for evaluating oncolytic viruses**
P330	Oncolytic viruses	S A McDade	*Merck KGaA (BioReliance Ltd)*	**The application of rapid molecular methods to overcome challenges in biosafety testing of oncolytic virus products**
P331	Oncolytic viruses	J Burke	*CG Oncology, Irvine, CA, USA*	**CG0070 for the treatment of non-muscle invasive bladder cancer (NMIBC) unresponsive to Bacillus Calmette-Guerin (BCG)**

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OTHER

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P332	Other	N L Yozwiak	*Ring Therapeutics*	**Anelloviruses are highly diverse, ubiquitous commensal denizens of the human virome, and show promising properties as novel gene delivery vectors**
P333	Other	B Handyside	*BioMarin Pharmaceutical*	**Effect of prophylactic corticosteroid treatment on adeno-associated virus (AAV)-mediated gene expression**
P334	Other	A Lasarte-Cía	*Universidad de Navarra*	**Antitumor effect of a self-amplyfing RNA expressing interleukin-12 in combination with in vivo electroporation**
P335	Other	R V Bell	*Imperial College London*	**Intravenous administration of F/HN pseudotyped lentiviral vector**

P336	Other	M Di Scala	*SpliceBio, C/Baldiri Reixach 10, Barcelona 08028, Spain.*	**Next generation split intein-based protein splicing to reconstitute large genes for gene therapy.**
P337	Other	E R Young	*UCL Institute of Child Health*	**Delivery of disease-responsive mini-intronic plasmids with cationic liposome vectors for rheumatoid arthritis**
P338	Other	I Vázquez-Domínguez	*Department of Human Genetics, Radboud university medical center, Nijmegen (The Netherlands)*	**Comparison of the exon skipping efficacy of different chemically modified antisense oligonucleotides within the mouse retina**
P339	Other	C Iglesias-Lopez	*Universitat Autònoma de Barcelona*	**Clinical and regulatory strategies to support the marketing authorisation of regenerative medicine products in Japan**
P340	Other	L S Chernova	*Kazan Federal University*	**The small heat shock protein AllbpA from mycoplasma Acholeplasma laidlawii prevents the formation of amyloid structures**
P341	Other	J Venkatas	*University of KwaZulu-Natal*	**Curcumin-capped Poly-L-Lysine modified gold nanoparticles for delivery of mRNA to cervical cancer cells**
P342	Other	E V Rozhina	*Kazan Federal University*	**Influence of monthmorillonite adsorbents on the efficacy of removing of pharmaceuticals from water**
P344	Other	J I Solomun	*Laboratory of Organic and Macromolecular Chemistry (IOMC), Friedrich Schiller University Jena*	**pH-responsive multicomponent system for charge masking of cationic nanoparticles for gene delivery**
P345	Other	F Richter	*Laboratory of Organic and Macromolecular Chemistry (IOMC), Friedrich Schiller University Jena, Germany*	**Optimizing polymeric micelles for non-viral pDNA delivery**

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PRIMARY IMMUNODEFICIENCIES

No.	Category	Speaker	Speaker Affiliation	Poster Title
P346	Primary immuno-deficiencies	M E Seker	*Hacettepe University*	**Using bone marrow permeabilisation instead of chemotherapy as a conditioning regimen for HSC gene therapy of RAG2-SCID**
P347	Primary immuno-deficiencies	C Iglesias-Herrero	*Laboratory for Molecular Virology and Gene Therapy, Department of Pharmaceutical and Pharmacological Sciences, KU Leuven*	**Ex vivo proof-of-concept study evaluating STAT1 gain-of-function gene therapeutic approaches**
P348	Primary immuno-deficiencies	Ö D Erol	*Hacettepe University*	**Correction of RAB27A gene from Griscelli Syndrome Type II derived Mesenchymal Stem Cells**

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SAFETY				
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P349	Safety	S Sheridan	*Merck KGaA*	**Bridging the gap: use of rapid quality control test methods for cell and gene therapies as alternatives to traditional methods**
P350	Safety	M Themis	*3. Brunel University London, Department of Life Sciences, Uxbridge, United Kingdom*	**Application of InGetox to assess the genotoxicity of LV and AAV vectors**
P351	Safety	C Colleoni	*Telethon Institute of Gene Therapy (HSR-TIGET)*	**Dissecting the non-cell-autonomous effects of oncogene activation on hematopoiesis**
P352	Safety	S Scott	*Commonwealth Scientific and Industrial Research Organisation*	**Finding integrations of a clinical gene therapy vector in NGS data with isling**
P353	Safety	D Glow	*Research Dept. Cell and Gene Therapy; Dept. of Stem Cell Transplantation, University Medical Centre Hamburg-Eppendorf, Hamburg (Germany)*	**LATE – a novel sensitive cell-based assay for the study of CRISPR-Cas9 related Long-term Adverse Treatment Effects**
P354	Safety	A L Bastone	*Institute of Experimental Hematology, Hannover Medical School*	**Development of a new all-in-one in vitro safety assay for gene therapy to detect lymphoid and myeloid insertional mutants**
P355	Safety	F Benedicenti	*San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)*	**Sonication Linker Mediated-PCR (SLiM-PCR), an efficient method for quantitative retrieval of vector integration sites**
P356	Safety	M Franco	*Genewerk GmbH Heidelberg*	**Qualification of an NGS-based assay for the identification and quantification of encapsidated sequences in rAAV batches**
P357	Safety	G Corre	*Genethon*	**Standardization of lentiviral vector copy quantification and vector insertion site detection using cellular clones.**
P358	Safety	S Suleman	*Department of Life Sciences, College of Health, Medicine & Life Sciences, Brunel University London, Uxbridge, UK*	**Lentivirus tethering to the genome is associated with transcription factor binding sites that favour virus survival**
P359	Safety	M Zahn	*GeneWerk GmbH Heidelberg*	**Standardization and Validation of a Probe-Based qPCR Assay for Lentiviral Vector Copy Number Determination**
P360	Safety	M Thomas	*Royal Perth Hospital, WA 6000, Australia*	**Safety Profile of Conditioning Regimens of AVROBIO's Investigational ex vivo Lentiviral Gene Therapy for Fabry Disease**

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STEM CELLS & REGENERATIVE MEDICINE				
No.	**Category**	**Speaker**	**Speaker Affiliation**	**Poster Title**
P361	Stem cells & regenerative medicine	B W Bigger	*The University of Manchester*	**Clinical Trial Update: Ex-vivo autologous haematopoietic stem cell gene therapy in MPSIIIA**
P362	Stem cells & regenerative medicine	D S Chulpanova	*Kazan Federal University*	**Artificial membrane vesicles isolated from mesenchymal stem cells with IL2 overexpression activate CD8+ T-killers to kill triple negative breast cancer cells**
P363	Stem cells & regenerative medicine	C Baricordi	*AVROBIO Inc., Cambridge MA*	**High throughput characterization of CD34+ cell states and dynamics upon genetic engineering**
P364	Stem cells & regenerative medicine	S Ferrara	*MeiraGTx*	**KCNV2 retinal organoid disease model for KCNV2 AAV gene therapy development**
P365	Stem cells & regenerative medicine	R Alatrash	*Kazan Federal University*	**Microvesicles transduced with NGF ameliorate pathological outcomes of EAE mouse model**
P366	Stem cells & regenerative medicine	M Gómez-Ferrer	*Grupo de Regeneración y Trasplante Cardiaco, Instituto de Investigacion sanitaria La Fe, Valencia, Spain*	**EVs derived from HIF-overexpressing MSC primed with a pro-inflammatory cocktail promote M2 macrophage polarization and reduce fibrosis and endothelium activation in vitro and in a mouse ulcerous colitis model.**
P367	Stem cells & regenerative medicine	E Zonari	*San Raffaele Telethon Insititute for Gene Therapy (SR-TIGET)*	**Clonal analysis of ex vivo expanded mobilized peripheral blood hematopoietic stem and progenitor cells in xenografts proves symmetric HSC division in culture**
P368	Stem cells & regenerative medicine	M Casini	*Instituto de Investigacion sanitaria La Fe*	**Subtype-specific differentiation of atrial and ventricular cardiomyocytes from human iPSCs**
P369	Stem cells & regenerative medicine	S S Issa	*Kazan (Volga Region) Federal University*	**Development of cell-mediated gene therapy method for metachromatic leukodystrophy using modified mesenchymal stem cells**
P370	Stem cells & regenerative medicine	A Lieske	*Hannover Medical School*	**Lentiviral fluorescent genetic barcoding allows real-time fate tracking of different hematopoietic stem cell populations in vivo**
P371	Stem cells & regenerative medicine	E Koçak	*Hacettepe University*	**PKNOX2 can be a new player in NF-κB/p65 signaling in bone marrow mesenchymal stem cell senescence**
P372	Stem cells & regenerative medicine	D S Chulpanova	*Kazan Federal University*	**The influence of cytochalasin B-induced membrane vesicles derived from mesenchymal stem cells overexpressing TRAIL on the expression of apoptotic genes in triple-negative MDA-MB-231 breast cancer cells**
P373	Stem cells & regenerative medicine	S M Abdin	*Clinic for Pediatric Pneumology, Allergology and Neonatology, Hannover Medical School, 30625Hannover, Germany*	**Fully defined iPSC-macrophages to assess pyrogens in parenteral drugs and medical products**

P374	Stem cells & regenerative medicine	R Vuerich	*Cardiovascular Biology Laboratory, International Centre for Genetic Engineering and Biotechnology (ICGEB), 34149 Trieste, Italy*	**Stromal Vascular Fraction scaffold promotes revascularization of ischemic non-healing wounds through direct cell integration and paracrine signals.**
P375	Stem cells & regenerative medicine	C Cugno	*Sidra Medicine, Doha, Qatar*	**IFNg-mediated immunomodulation on Mesenchymal Stromal Cells is driven by genetic and epigenetic mechanisms**
P376	Stem cells & regenerative medicine	S V Kurbangaleeva	*Kazan Federal University*	**Stem cell-derived microvesicles transfer surface receptors to epithelial cells**
P377	Stem cells & regenerative medicine	U D Dyachkova	*Faculty of Medicine, Lomonosov Moscow State University, Moscow, Russia*	**M2-macrophage-induced chronic inflammation promotes mesenchymal stromal cell senescence.**
P378	Stem cells & regenerative medicine	E A Naumenko	*Kazan Federal University*	**Osteoconductive properties of nanomodified scaffolds**
P379	Stem cells & regenerative medicine	R González-Sastre	*Instituto de Salud Carlos III*	**Improving the generation of human brain organoids from pluripotent stem cells**
P380	Stem cells & regenerative medicine	B J Critchley	*UCL Institute of Child Health, London, UK*	**Targeting the blood-brain barrier to increase haematopoietic stem cell engraftment for neurological lysosomal storage disease gene and cell therapy**
P381	Stem cells & regenerative medicine	I Reinal	*Instituto de Investigacion sanitaria La Fe*	**Characterization of hiPSC-CMs derived from pediatric cancer patients treated with anthracyclines that suffered cardiotoxicity**
P382	Stem cells & regenerative medicine	L Barrachina	*Laboratorio de Genética Bioquímica LAGENBIO (Universidad de Zaragoza), Instituto Agroalimentario de Aragón– IA2 - (Universidad de Zaragoza-CITA); Instituto de Investigación Sanitaria de Aragón (IIS), Zaragoza, 50013, Spain*	**Immunomodulatory capacity of equine mesenchymal stem cells (MSCs) is influenced by inflammation, differentiation and compatibility for the major histocompatibility complex (MHC)**
P383	Stem cells & regenerative medicine	A Rosca	*Instituto de Salud Carlos III*	**Effects of chlorpyrifos on stem cell biology: cell death and phenotypic specification**
P384	Stem cells & regenerative medicine	Z E Gilazieva	*Kazan Federal University*	**Evaluation of induced membrane vesicles effect on tumor spheroids**

P385	Stem cells & regenerative medicine	A A Shaimardanova	*Kazan Federal University*	**Development of cell-mediated gene therapy for Tay-Sachs disease**
P386	Stem cells & regenerative medicine	A Cequier	*Laboratorio de Genética Bioquímica LAGENBIO (Universidad de Zaragoza), Instituto Agroalimentario de Aragón– IA2 - (Universidad de Zaragoza-CITA); Instituto de Investigación Sanitaria de Aragón (IIS), Zaragoza, 50013, Spain*	**The cellular immune response to allogeneic equine mesenchymal stem cells (MSCs) induces changes in their immunomodulatory and immunogenic profiles**
P387	Stem cells & regenerative medicine	F Marinaro	*Centro de Cirugía de Mínima Invasión Jesús Usón*	**Bilayer fibrin-coated surgical meshes seeded with menstrual-blood derived stromal cells show immunomodulatory properties in vitro**
P388	Stem cells & regenerative medicine	M C Arufe	*Universidade da Coruña*	**Therapy free of cells vs human mesenchymal stem cells from umbilical cord strome. Effect of miR21 to treat the inflammation in Osteoarthritis.**
P389	Stem cells & regenerative medicine	E V Rozhina	*Kazan Federal University*	**Three-dimensional hepatocellular cultures for modeling diseases and assessing the efficacy of drugs and their hepatotoxicity**
P390	Stem cells & regenerative medicine	S S Michurina	*NMRC of Cardiology, Moscow*	**Developing of glucose-consuming adipocytes by interleukin 4 gene delivery**
P391	Stem cells & regenerative medicine	G Seker	*The Department of Gene and Cell Therapy, Akdeniz University, Faculty of Medicine, Antalya, 07058, Turkey*	**Successful generation of induced pluripotent stem cells from HEK-293T cells via sodium butyrate and codon-optimized mini intronic plasmids encoding reprogramming factors**
P392	Stem cells & regenerative medicine	A Bettini	*University College London*	**Optimising culture of iPSC-derived cardiomyocytes for delivery into the heart**
P393	Stem cells & regenerative medicine	I Galasso	*Telethon Institute of Gene Therapy (HSR-TIGET)*	**In vivo and in vitro optimization of protocols for the expansion of genetically-engineered human hematopoietic stem and progenitor cells**
P394	Stem cells & regenerative medicine	R Coronel	*Instituto de Salud Carlos III*	**Role of Amyloid Precursor Protein (APP) in the Cell Fate Specification of Human Neural Stem Cells**
P395	Stem cells & regenerative medicine	A Naeem	*Meira GTx UK II, 34-38 Provost Street, London N1 7NG*	**GUCY2D retinal organoid disease model for AAV gene therapy development**

P396	Stem cells & regenerative medicine	D A Fedotov	*Faculty of Medicine, Lomonosov Moscow State University, Moscow, Russia*	**Reverse of mesenchymal stromal cell senescence by senolytics in vitro.**
P397	Stem cells & regenerative medicine	K V Kitaeva	*Kazan Federal University*	**The proliferative activity and cytokine secretion changes in mesenchymal stem cell and neuroblastoma cells during co-culture under hypoxic conditions**
P398	Stem cells & regenerative medicine	M Á de Pedro	*Jesús Usón Minimally Invasive Surgery Centre, Cáceres, 10071, Spain*	**Effect of short-term Hypoxic Culture Conditions on Menstrual Blood Stromal cells**
P399	Stem cells & regenerative medicine	S Rezaeiani	*1- Department of Stem Cells and Developmental Biology, Cell Science Research Center, Royan Institute for Stem Cell Biology and Technology, ACECR, Tehran, 1665659911, Iran.*	**Prediction of a surface marker for cardiac progenitor cells derived from human pluripotent stem cells Prediction of a surface marker for cardiac progenitor cells derived from human pluripotent stem cells**
P400	Stem cells & regenerative medicine	J C Buitrago	*Curexsys GmbH, Göttingen, 37079, Germany*	**Differentially expressed miRNAs in human Wharton's Jelly mesenchymal stromal cells-derived extracellular vesicles: revealing potential mechanisms of action in immune modulation**
P401	Stem cells & regenerative medicine	E Amaro-Prellezo	*Grupo de Regeneración y Trasplante Cardíaco, Instituto de Investigación Sanitaria La Fe, Valencia, 46026, España*	**Isolation of extracellular vesicles derived from mesenchymal stem cells by ultracentrifugation and tangential flow filtration followed by size-exclusion chromatography: a functional comparison**

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