

Poster List

P01 CLEAN: a universal platform for seamless targeted gene addition by self-enforcing saturating selection in human hematopoietic stem cells Alessandra Weber, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy.</i>	Emerging tools and cell engineering approaches
P02 Molecular Radio-Cell Theranostic Platforms Integrating Molecular Imaging and Adoptive Cell Immunotherapy Anna Gaimari, <i>Istituto Romagnolo per lo Studio Dei Tumori "Dino Amadori" - IRST IRCCS</i>	Emerging tools and cell engineering approaches
P03 Antigen-specific dendritic cell immunotherapy to halt autoimmunity in Type 1 Diabetes Aurora Forlani, <i>Università Vita-Salute San Raffaele, Milan San Raffaele Institute for Gene Therapy - SR-TIGET</i>	Emerging tools and cell engineering approaches
P04 Baboon retrovirus envelope pseudotyped lentiviral vectors outperform VSVG pseudotyped vectors for macrophage transduction efficacy, cell survival and conservation of phenotype Chiara Martinello, <i>Université Côte d'Azur</i>	Emerging tools and cell engineering approaches
P05 Developing CRISPRa-engineered human induced pluripotent stem cell-derived neural stem cells (hiPSC-NSCs) to empower therapeutic strategies for demyelinating diseases Chiara Garsia, <i>Gene and Neural stem cell therapy for lysosomal storage diseases, SR TIGET - The San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy Vita-Salute San Raffaele University, Milan, Italy</i>	Emerging tools and cell engineering approaches
P06 Targeted and saturating ABCD1 Gene Addition in HSPCs for the treatment of X-linked adrenoleukodystrophy Damiano Pulito, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy</i>	Emerging tools and cell engineering approaches
P07 Dual-vector gene editing approach for precision immunoengineering and targeted payload integration Eleonora Carito, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, 20132, Italy Vita-Salute San Raffaele University, Milan, 20132, Italy</i>	Emerging tools and cell engineering approaches
P08 Hypoimmunogenic engineered lentiviral vectors to escape in vivo immune recognition Giulia Buccarello, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Hospital, Milan, Italy</i>	Emerging tools and cell engineering approaches
P09 Targeted gene addition in human HSPCs using TILV for protein replacement therapies Giulia Scalisi, <i>Genethon Inserm UMR951</i>	Emerging tools and cell engineering approaches
P10 Antigen-specific IL-10-engineered dendritic cells modulate autoimmune neuroinflammation in neuromyelitis optica Gloria Giacomello, <i>1Mechanisms of Peripheral Tolerance, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan; 2 DIMET PhD Program, Università Milano Bicocca, Milan; 3Clinical Neuroimmunology Unit, Institute of Experimental Neurology, Division of Neuroscience, IRCCS Ospedale San Raffaele, and Vita e Salute San Raffaele University, Milan; 4Neurology Unit, Neurorehabilitation Unit, and Neurophysiology Service, and Neuroimaging Research Unit, Division of Neuroscience, IRCCS San Raffaele Scientific Institute, Milan</i>	Emerging tools and cell engineering approaches
P11 Guido of a comprehensive HSPC atlas across sources and ages Guido Pacini, <i>San Raffaele Telethon Institute for Gene Therapy</i>	Emerging tools and cell engineering approaches
P12 Computational identification and experimental validation of targetable HSC-niche retention pathways to improve genotoxic-free hematopoietic stem cell gene therapy Hussein Najib Antar, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, 20132 Milan (MI), Italy Vita-Salute San Raffaele University, 20132 Milan (MI), Italy</i>	Emerging tools and cell engineering approaches
P13 Novel Chimeric Antigen Receptor T cell therapy targeting mislocalised cell surface HSP90B1 for glioblastoma and other solid tumours Krishneel Prasad, <i>Walter and Eliza Hall Institute of Medical Research, Parkville 3052, Australia Department of Medical Biology, The University of Melbourne, Parkville 3052, Australia</i>	Emerging tools and cell engineering approaches
P14 Designing novel Versatile Proteolysis Chimeric Antigen Receptor (VIPER) T cell therapies for glioma and other solid tumours Krishneel Prasad, <i>Walter and Eliza Hall Institute of Medical Research, Parkville 3052, Australia Department of Medical Biology, The University of Melbourne, Parkville 3052, Australia</i>	Emerging tools and cell engineering approaches
P15 Designing Synthetic Intramembrane Proteolysis Receptors (SNIPRs) for parallel gene induction in T cells Krishneel Prasad, <i>Walter and Eliza Hall Institute of Medical Research, Parkville 3052, Australia Department of Medical Biology, The University of Melbourne, Parkville 3052, Australia</i>	Emerging tools and cell engineering approaches
P16 Lentiviral Vector-Mediated UBA1 Correction as a therapeutic approach for VEXAS syndrome Laura Alessandrini, <i>San Raffaele Telethon Institute for Gene Therapy Vita-Salute San Raffaele University</i>	Emerging tools and cell engineering approaches
P17 Investigating the biological properties of circulating Hematopoietic Stem Progenitor Cells in paediatric subjects as novel source for Gene Therapy. Luca Seffin, <i>San Raffaele Telethon Institute for Gene Therapy</i>	Emerging tools and cell engineering approaches
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P19 Lipid Nanoparticles Enable Efficient and Scalable Ex Vivo Gene Editing in HSPCs Martina Fiumara, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy. Vita-Salute San Raffaele University, Milan, Italy.</i>	Emerging tools and cell engineering approaches
P20 Metabolic rewiring of DC by IL-10: decoding the pathways governing immunoregulatory function Michela Vuono, <i>Mechanisms of Peripheral Tolerance Unit, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy PhD Course in Molecular Medicine, Vita-Salute San Raffaele University, Milan, Italy</i>	Emerging tools and cell engineering approaches
P21 HER2-CAR-T therapy eradicates drug-tolerant persisters in ALK-rearranged lung cancer organoids and xenograft models Francesca Picca, <i>Department of Oncology, University of Torino, Orbassano, Italy Department of Molecular Biotechnology and Health Sciences, University of Torino, Torino, Italy</i>	Emerging tools and cell engineering approaches
P22 CRISPRa-mediated utrophin upregulation as a universal therapy for DMD Paola Galbiati, <i>Genethon, Evry, 91000, France.</i>	Emerging tools and cell engineering approaches
P23 In vivo macrophage engineering to deliver new immune-activating molecules to liver metastases Sara Breggion, <i>Vector Engineering and In Vivo Tumor Targeting Unit, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan 20132, Italy Vita-Salute San Raffaele University, Milan 20132, Italy</i>	Emerging tools and cell engineering approaches
P24 Computational and targeted CRISPR screening in HSPCs identifies haploinsufficient genes governing RNA processing, translation, and transcription as selection and conditioning candidates Vigneshwaran Venkatesan, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy.</i>	Emerging tools and cell engineering approaches
P25 A customizable extracellular vesicle platform for selective CRISPR Cas9 delivery and gene editing Alessio Ferraro, <i>Istituto nazionale di genetica molecolare INGM</i>	Emerging tools and cell engineering approaches
P26 Novel envelope glycoproteins for lentiviral vector pseudotyping enable efficient and selective target cell transduction Daria Saibene, <i>Vector Engineering and In Vivo Tumor Targeting Unit, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Hospital, Milan, Italy Vita-Salute San Raffaele University, Milan, Italy</i>	Emerging tools and cell engineering approaches
P27 Gene-sized editing with a multiplexable polyfunctional editor allows selection of precisely edited hematopoietic stem cells with enhanced engraftment potential Dario Russo, <i>San Raffaele Telethon Institute for Gene Therapy University School of Advanced Studies IUSS</i>	Emerging tools and cell engineering approaches
P28 Brain-targeted in vivo base editing for correction of Krabbe disease Elena Pelloni, <i>Center for Genomic Medicine and Rare Diseases, Department of Medical and Surgical Sciences, University of Modena and Reggio Emilia, Modena, Italy</i>	Emerging tools and cell engineering approaches
P29 Naturally occurring AAV serotype enables efficient retinal transduction Emanuela Pone, <i>Gene Therapy Joint Lab</i>	Emerging tools and cell engineering approaches
P30 A new generation of lentiviral vectors to enable precise temporal and spatial control of transgene expression Matilde Bellandi, <i>Vita-Salute San Raffaele University, Milan 20132, Italy</i>	Emerging tools and cell engineering approaches
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P32 A vesicle-based platform for high-efficiency, high-viability CRISPR Cas9 knockout in primary human myeloid cells Silvia Fiori, <i>Istituto Nazionale Genetica Molecolare (INGM) Università degli Studi di Milano (UNIMIL), Milan, Italy</i>	Emerging tools and cell engineering approaches
P33 CRISPR-based functional genomics for rAAV production Tommaso Ferrari, <i>University of Modena and Reggio Emilia</i>	Emerging tools and cell engineering approaches
P34 Targeting the bone marrow niche by FGF23 inhibition improves the engraftment of transduced hematopoietic stem cells in a preclinical model of gene therapy for beta-thalassemia Annamaria Aprile, <i>Gene Transfer into Stem Cells Unit, San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy</i>	Gene correction strategies and clinical trials updates
P35 Myeloid-mediated enzymatic correction of ARSA-deficient neural cells inhematopoietic stem cell gene therapy for metachromatic leukodystrophy Filippo Casalini, <i>1. San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS Ospedale San Raffaele, 20132 Milan, Italy</i>	Gene correction strategies and clinical trials updates
P36 Immune regulation of hematopoietic stem cell fitness and commitment in Beta -thalassemia Giulia Chianella, <i>San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), IRCCS San Raffaele Scientific Institute Milan, Italy</i>	Gene correction strategies and clinical trials updates
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P38 Optimized transduction of mPB CD34+ cells increase transduction efficiency and VCN while preserving stemness: towards a Phase Ib clinical trial of gene therapy for TDT beta-thalassemia Samantha Scaramuzza, <i>OSR TIGET</i>	Gene correction strategies and clinical trials updates
P39 Targeting IL1 signaling improves HSC function and MSC support to HSC in Sickle Cell Disease Silvia Sighinolfi, <i>San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan Italy</i>	Gene correction strategies and clinical trials updates
P40 Preclinical development of lentiviral-based hematopoietic stem progenitor cells (HSPC)-gene therapy (GT) for Mucopolysaccharidosis type IVA within an innovative platform program for Lysosomal Storage Disorders Stefania Crippa, <i>San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy</i>	Gene correction strategies and clinical trials updates
P41 An innovative platform approach for the parallel development of HSPC-GT for rare ultra-rare lysosomal storage disorders with severe skeletal and neurological manifestations. Stefania Crippa, <i>San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy</i>	Gene correction strategies and clinical trials updates
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P44 Lentiviral vector-mediated in vivo platelet FVIII gene therapy for Hemophilia A Cecilia Ianni, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, 20132, Italy</i>	In vivo pre-clinical gene therapy
P45 Gene therapy enables durable hematopoietic reconstitution in deficiency of adenosine deaminase 2 Chiara Rigamonti, <i>Mechanisms of Inflammation in Health and Disease Unit, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy Vita-Salute San Raffaele University, Milan, Italy</i>	In vivo pre-clinical gene therapy
P46 AAV integration, vector–host splicing and clonal selection shape the therapeutic effect of liver gene replacement and genome editing Elisa Bonomi, <i>San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), IRCCS San Raffaele Scientific Institute, Milan, Italy Department of Electronics, Information and Bioengineering, Politecnico di Milano, Milan, Italy</i>	In vivo pre-clinical gene therapy
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P52 Challenges of liver-directed gene therapy for glycogen storage disease Type 3 Luca Bocchialini, <i>Généthon, Évry, France Paris-Saclay University, Evry University, Inserm, Généthon, Integrare Research Unit UMR_S951, Évry, France</i>	In vivo pre-clinical gene therapy
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P55 Overcoming toxic conditioning in hematopoietic stem cell gene therapy leveraging mobilization-resistant CXCR4 variants Ottavia Vitaloni, <i>San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, 20132 Milan (MI), Italy</i>	In vivo pre-clinical gene therapy
P56 Pre-clinical development of an ex-vivo Hematopoietic Stem progenitor Cells-gene therapy for a-Mannosidosis Rachele D'Amore, <i>San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), San Raffaele Scientific Institute, Milan, Italy Università Vita-Salute San Raffaele, Milan, Italy</i>	In vivo pre-clinical gene therapy
P57 Engineering thymic selection to promote acceptance of allogeneic transplantation. Valentina Consoli, <i>San Raffaele Telethon Institute for gene therapy (SR-Tiget), Milan, Italy</i>	In vivo pre-clinical gene therapy
P58 Genome editing for therapy of Leber Congenital Amaurosis type 10 Daria Ponomareva, <i>Tigem - Telethon Institute of Genetics and Medicine University School of Advanced Studies IUSS, Pavia</i>	In vivo pre-clinical gene therapy
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P60 Targeting white matter astrocytes with a Ggap-disruptive Cas9 nuclease to treat Alexander disease Irene Capuano, <i>San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Gene Neural stem cell therapy for lysosomal storage diseases; Milan, Italy.</i>	In vivo pre-clinical gene therapy
P61 Preclinical development of an optimized bicistronic lentiviral vector enabling effective HSC gene therapy for GM2 gangliosidosis Irene Giustolisi, <i>San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS Ospedale San Raffaele, 20132 Milan, Italy Università Vita Salute San Raffaele (UniSR), 20132 Milan, Italy</i>	In vivo pre-clinical gene therapy
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P68 Long reading going short: Oxford Nanopore direct RNA sequencing of synthetic guide RNA Francesca Ferrara, <i>St. Jude Vector Laboratory, St. Jude Children's Research Hospital, Memphis, TN, United States</i>	Safety and responses to gene therapy