Programme Overview

08:00 - 09:00	**Registration desk opens**. Welcome tea & coffee
08:30 - 09:00	**BSGCT Annual General Meeting** (members only) Auditorium
09:00 - 10:30	**Session 1 - Technology overview and Fairbairn Awards** Chairs: Uta Griesenbach & Aarash Saleh Auditorium
09:00 - 09:30	**INV01** Lentiviral Vectors Gene Therapy: from bench to bedside and back Professor Luigi Naldini, Head of Unit SR-Tiget, Italy
09.30 - 09:45	**FB01** Gp100-TCRs from TIL for targeted melanoma treatment Natasha Myhill, University Of Manchester
09.45 - 10.00	**FB02** Lentivirus GM-CSF gene therapy for autoimmune pulmonary alveolar proteinosis Dr Helena Lund-Palau, Imperial College London
10.00 - 10.15	**FB03** Intravenously administered Gene therapy For neuronopathic gaucher disease Dr Giulia Massaro, UCL
10.15 - 10.30	**FB04** Insights into Species D Adenovirus receptor usage from structural characterisation Alexander Baker, Cardiff University
10:30 - 11:00	**Morning tea break, exhibition and posters** Exhibition, poster and catering areas
11:00 - 12:15	**Session 2 - In vivo and ex vivo applications and selected poster presentations** Chairs: Alexander Baker & Mimoun Azzouz Auditorium Sponsored By: Merck BioReliance® Services
11:00 - 11:30	**INV02** Gene therapy for primary immunodeficiencies Claire Booth, UCL Great Ormond Street Institute of Child Health
11:30 - 12:00	**INV03** Lentiviral vectors for in vivo gene therapy Dr Kyriacos Mitrophanous, Oxford BioMedica
12:00 - 12:15	**1 minute rapid poster presentations** (PO09-PO18) **RPO09** AAV9 intracerebroventricular gene therapy improves lifespan and normalises long-term locomotor behaviour in a mouse model of Niemann-Pick type C1 disease Dr Michael Hughes, University College London **RPO10** Lentiviral gene therapy for p22phox deficient chronic granulomatous disease Andrei Claudiu Cozmescu, University College London **RPO11** AAV gene therapy for Dravet Syndrome Juan Antinao Díaz, UCL **RPO12** Large transgene strategies for von Willebrand disease Robyn Bell, Imperial College London **RPO13** Enhancing peripheral nerve regeneration through combined gene therapy and tissue engineering Francesca Busuttil, UCL

	RPO14 A method for assessing the distribution of eGFP expressing cells in the mouse lung following lentiviral vector transduction Rosie Munday, University Of Oxford **RPO15** Oscillating magnetic fields post-transfection can promote endosomal escape and enhanced transgene expression for GET-mediated transfection Lia Andrea Blokpoel Ferreras, University Of Nottingham **RPO16** Bioprocessing and engineering characterisation of T-cell therapy manufacture in an ambr® 250 bioreactor Elena Costariol, University College London **RPO17** Minimising the A2UCOE for direct transgene expression from the innate HNRPA2B1 promoter Omer Faruk Anakok, Academician Ataturk University **RPO18** Validation of a PCR-based assay to quantify lentiviral vector shedding in human body fluids Nora Clarke, Imperial College London
12:15 - 13:25	**Lunch, exhibition and posters** (all even numbers 12:15-12:45, all odd numbers 12:45-13:15) **PO01** Precision Cut Lung Slices (PCLSs) as a model for testing lung gene therapies Dr Gizem Osman, University Of Nottingham **PO02** Large-scale, high-throughput production of lentiviral vectors for multiple disease applications Rachel Ashworth, University Of Oxford **PO03** Abstract Withdrawn **PO04** Expression profile of optimised SIV vector in the mouse lung at early timepoints post-delivery Dr Ian Pringle, University Of Oxford **PO05** Investigation of repeat viral vector administration through immune tolerance Joost van Haasteren, University Of Oxford **PO06** RNA in-situ hybridisation is able to quantify lentiviral transduction of respiratory epithelium Dr Aarash Saleh, Imperial College London **PO07** Characterizing virus preparations using Nanoparticle Tracking Analysis (NTA) – Adenovirus case study Pauline Carnell-Morris, Malvern Panalytical Ltd **PO08** Enhancing the generation of CD8+ CAR-T central memory cells Arman Amini, University College London **PO09** AAV9 Intracerebroventricular Gene Therapy Improves Lifespan and Normalises Long-term Locomotor Behaviour in a Mouse Model of Niemann-Pick type C1 Disease Dr Michael Hughes, University College London **PO10** Lentiviral gene therapy for p22phox deficient chronic granulomatous disease Andrei Claudiu Cozmescu, University College London **PO11** AAV gene therapy for Dravet Syndrome Juan Antinao Díaz, UCL **PO12** Large transgene strategies for von Willebrand disease Robyn Bell, Imperial College London

12:15 - 13:25	**Lunch, exhibition and posters** (continued) (all even numbers 12:15-12:45, all odd numbers 12:45-13:15) **PO13** Enhancing peripheral nerve regeneration through combined gene therapy and tissue engineering Francesca Busuttil, UCL **PO14** A method for assessing the distribution of eGFP expressing cells in the mouse lung following lentiviral vector transduction Rosie Munday, University Of Oxford **PO15** Oscillating magnetic fields post-transfection can promote endosomal escape and enhanced transgene expression for GET-mediated transfection Lia Andrea Blokpoel Ferreras, University Of Nottingham **PO16** Bioprocessing and engineering characterisation of T-cell therapy manufacture in an ambr® 250 bioreactor Elena Costariol, University College London **PO17** Minimising the A2UCOE for direct transgene expression from the innate HNRPA2B1 promoter Omer Faruk Anakok, Academician Ataturk University **PO18** Validation of a PCR-based assay to quantify lentiviral vector shedding in human body fluids Nora Clarke, Imperial College London **PO19** A microfluidic device for flexible automated cell transduction John Bridgeman, Cellular Therapeutics **PO20** Phenotype and genotype characterization of Usher syndrome in Russian cohort (USHR) for potential inclusion in gene therapy trial Marianna Ivanova, Oftalmic Llc **PO21** Correction of p47-phox deficient Chronic Granulomatous Disease by lentiviral gene therapy Andrea Schejtman, UCL/ICH
13:30 - 15:00	**Session 3 - Challenges** Chairs: Claire Booth & Sophia-Martha Kleine Holthaus Auditorium
13:30 - 14:00	**INV04** Scale-up challenges -an academic view Steve Hyde, Oxford University
14:00 - 14:30	**INV05** Cell-mediated exon skipping for the therapy of Duchenne muscular dystrophy Professor Giulio Cossu, University of Manchester
14:30 - 15:00	**INV06** Quality of ATMPs – a European regulator's perspective Janet Glassford, Senior Quality Assessor MHRA **INV06** Regulatory challenges Ian Rees, Inspectorate Strategy and Innovation Unit MHRA
15:00 - 15:30	**Morning tea break, exhibition and posters** Exhibition, poster and catering areas

15:30 - 16:50	**Session 4 - Safety and novel vector design** Chairs: Giulio Cossu & Uta Luigi Naldini Auditorium
15:30 - 15:50	**INV07** Control & Standardisation, the manufacturing platform Keith Smith, NHS Blood & Transplant
15:50 - 16:10	**INV08** A unique cost effective humanised genotoxicity model bringing safety assessment to gene therapy Dr Michael Themis, Brunel University London
16:10 - 16:30	**INV09** From a cancer causing virus to a potentially safer strategy for gene therapeutics Professor Axel Schambach, Hannover Medical School
16:30 - 16:50	**INV10** Delivery of genome editing tool kits by lentiviral protein transduction' Professor Jacob Giehm Mikkelsen, Aarhus University
17:00 - 18:00	**Moderated Q&A - Pros and Cons of Gene Editing vs Gene Addition** Chairs: Brian Bigger & Tassos Georgiadis Panelists: Claire Booth, Professor John Harris, Professor Jacob Giehm Mikkelsen, Professor Luigi Naldini Auditorium
18:00 - 18:15	**Presentation of awards and close of conference** Auditorium
18:15 - 21:30	**Networking Drinks** Tuke Common Room Sponsored By: Synpromics Synpromics the leader in gene control

Disclaimer: Speaker names, affiliations, abstract titles and abstracts in the Programme are presented as submitted by the corresponding author.

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