Agenda

Wednesday 24 April 2024 | 09.00 – 17.30
Kennedy Lecture Theatre, UCL Great Ormond Street Institute of Child Health

Brought to you by the UCL Translational Research Office (TRO), which provides an invaluable opportunity for interaction with research and industry experts, fostering collaborations and connections within and beyond UCL.

08.30 – 09.00 Registration

09.00 – 09.10 Welcome and Introductions

Professor Simon Waddington, Professor of Gene Therapy, Maternal & Fetal Medicine, UCL; Chair of UCL Therapeutic Innovation Networks (TINs) – Cell and Gene Therapy

09.10 – 11.10 Session 1 – UCL Advanced Therapy Successful Case Studies

Chair: Professor Simon Waddington, Professor of Gene Therapy, Maternal & Fetal Medicine, UCL; Chair of UCL Therapeutic Innovation Networks (TINs) – Cell and Gene Therapy

9.10 – 9.40 **Professor Emma Morris**, Professor of Clinical Cell and Gene Therapy, UCL Institute of Immunity & Transplantation; Director, UCL Division of Infection and Immunity, UCL; Consultant Haematologist, Haematopoietic Stem Cell Transplantation; Lead, Adult Immunodeficiency HSCT and Gene Therapy Service

Gene editing for inborn errors of immunity

9.40 – 10.10 **Professor Paul Gissen**, Clinical Professor in Paediatric Metabolic Medicine and an Honorary Consultant at UCL Great Ormond Street Hospital for Children

Bloomsbury GTX/OTC case study

10.10 – 10:40 **Professor Bobby Gaspar**, Honorary Professor of Paediatrics and Immunology, UCL Great Ormond Street Institute of Child Health

Haematopoietic stem cell gene therapy – making genetic medicines

10.40 – 11.10 Panel Discussion 1 - Investment: Industry, VCs & Spinouts

Moderated by: Dr Anne Lane, CEO, UCL Business

Professor Emma Morris

Professor Bobby Gaspar

Dr Richard Fagan, Director of BioPharm, UCL Business

External guest – Dr Elisa Petris, Lead Partner, Syncona Investment Management Ltd

11.10 – 11.45 Networking Break & Exhibition

11.45 – 13.15 Session 2 – Early-stage Research

Chair: Dr Rajvinder Karda, Associate Professor in Gene Therapy, EGA Institute for Women's Health, UCL

11.45 – 12.15 **Professor Gabriele Lignani**, Professor of Translational Neuroscience, UCL

Gene therapy for epilepsy

12.15 – 12.45 **Dr Haiyan Zhou**, Associate Professor, Genetics & Genomic Medicine Department, UCL

Preserving sense by antisense

12.45 – 13.15 **Talks by Early Career Researchers**

Dr Stephanie Efthymiou, Research Fellow, Department of Neuromuscular Diseases, UCL
Towards generating an iPSc-based organoid model for characterising the NARS1 disease at a cellular level

Dr Annalucia Darbey, Research Fellow, Department of Neuromuscular Diseases, UCL
Developing a Muscle-Targeted Gene Therapy for Kennedy's Disease

Dr Amy McTague, Clinical Consultant - Research Fellow, Developmental Neurosciences Department, UCL
A patient-derived neuronal epilepsy model towards novel therapy discovery

13.15 – 14.15 Lunch, Networking, Exhibition & Poster Presentation – sponsored by UCL Business

14.15 – 15.45 Session 3 – Clinical Experience

Chair: Professor Dimitri Michael Kullmann, Professor of Neurology Clinical & Experimental Epilepsy, UCL Queen Square Institute of Neurology

14.15 – 14.45 **Professor Amit Nathwani**, Senior National Institute for Health Research (NIHR) Investigator and Professor of Haematology, UCL; Founder and CEO of NovalGen
Haemophilia gene therapy concept to market approval

14.45 – 15.15 **Professor Sarah Tabrizi**, Director of the UCL Huntington's Disease Centre; Joint Head of Department of Neurodegenerative Disease, UCL Queen Square Institute of Neurology; Principal Investigator, UK Dementia Research Institute; Honorary Consultant Neurologist, National Hospital for Neurology and Neurosurgery
Developing genetic therapies for Huntington's disease – challenges and opportunities

15.15 – 15.45 Panel Discussion 2 – UCL's clinician's experience

Professor Amit Nathwani

Professor Sarah Tabrizi

Professor Manju Kurian, Professor of Neurogenetics and NIHR Research Professor, UCL-Great Ormond Street Institute of Child Health

Professor Claire Booth, Mahboubian Professor in Gene Therapy and Paediatric Immunology, Head of Infection, Immunity and Inflammation Department, UCL; Deputy Theme Lead Gene, Stem and Cell Therapy, GOSH NIHR BRC; Cell & Gene Therapy Service Clinical Academic Lead; Consultant in Paediatric Immunology, Great Ormond Street Hospital

15.45 – 16.15 Networking Break & Exhibition

16.15 – 17.15 Session 4 – Cancer: CAR T-cell manufacture and therapies

Chair: Professor Daniel Bracewell, Professor of Bioprocess Analysis, Department of Biochemical Engineering, UCL

16.15 – 16.45 **Professor Qasim Rafiq,** Professor of Bioprocess Engineering, Department of Biochemical Engineering, UCL
Automated Biomanufacturing and Scale-up Strategies for ATMPs

16.45 – 17.15 **Dr Claire Roddie**, Consultant Haematologist, UCLH; Associate Professor in Haematology, UCL
Advances in CAR-T development at UCL

17.15 – 17.30 Closing Remarks

Professor Geraint Rees, Vice-Provost (Research, Innovation & Global Engagement), UCL

17.30– 19.00 Networking Reception