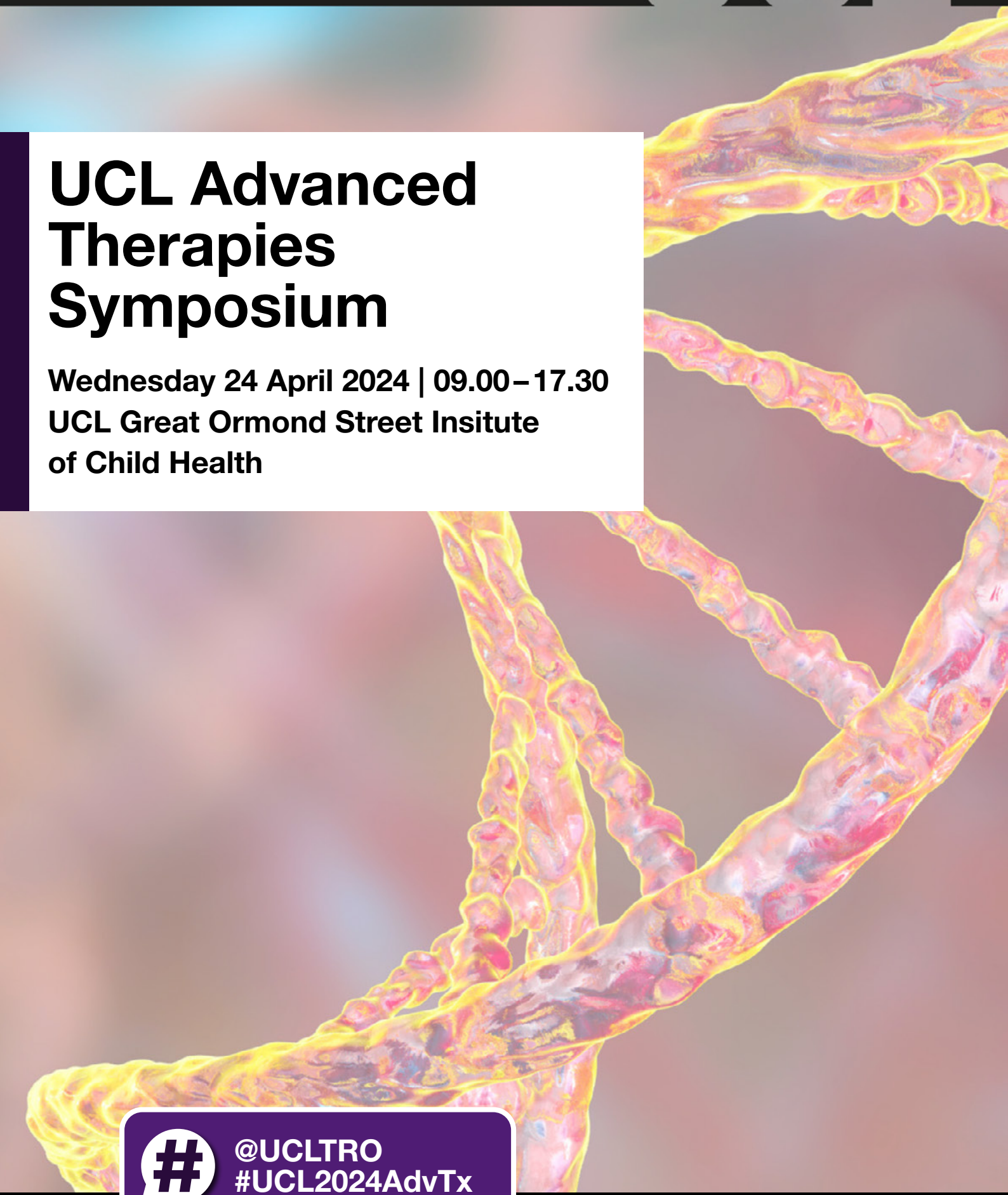
Translational Research Office (TRO)

UCL

UCL Advanced Therapies Symposium

Wednesday 24 April 2024 | 09.00–17.30
UCL Great Ormond Street Insitute of Child Health



@UCLTRO
#UCL2024AdvTx

Celebrating UCL's expertise in advanced therapies and its unique multidisciplinary approach, the inaugural UCL Advanced Therapies Symposium aims to bring together UCL scientists, research and clinical collaborators, funding entities, and healthcare industry leaders who specialise in this area.

Contents

Welcome

Dear Colleagues,

Welcome to the UCL Advanced Therapies Symposium, brought to you by the UCL Translational Research Office and the Therapeutic Innovation Networks (TINs).

Cell, gene, and regenerative therapies have been long-standing strengths of UCL, underpinned by over 30 years of public and privately funded research. UCL has one of the most extensive Advanced Therapy Medicinal Products (ATMP) pipelines in the world, with over 100 projects in development. Our extensive experimental and clinical expertise spans across disciplines, highlighting specialisms in Rare Diseases, Ophthalmology, Haematology/Oncology, Neurology, and Immunology. Over the past 10 years UCL has successfully spun out twelve advanced therapy companies, which collectively have received in excess of £2bn of external investment, with five companies having IPO'd.

Statistics* also show that 54% of all UK academic-sponsored clinical trials and 43% of all UK commercial-sponsored clinical trials are delivered by UCL partner hospitals, demonstrating our leading position in innovating and delivering these complex therapies.

This year, we are proud to host our first-ever advanced therapies symposium, highlighting UCL's excellence in advanced therapies across disciplines and the immense potential for translating research into innovative therapeutics for patient benefit and addressing real-life challenges.

Today's presentations will focus on:

1. **successful case studies;**
2. **early-stage research;**
3. **clinical experience; and**
4. **Cancer: CAR T-cell manufacture and therapies.**

Panel discussions promise to bring thought-provoking insights on **industry and VC investments in spinouts** as well as **clinical experience** in delivering these complex therapies.

Also, do not miss the poster presentation by our budding early career researchers who will showcase their work and inspiring ideas. We invite you to capitalise on the networking opportunity with UCL scientists, research and clinical collaborators, funding entities, and healthcare industry leaders to create new connections and stimulate partnership opportunities.

We would also like to thank the sponsors for making the event possible. Please visit the exhibition desks by our sponsoring partners during the breaks.

We hope you enjoy the event and look forward to interacting with you throughout the day.

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

* Data from Cell and Gene Therapy Catapult UK Clinical Trials Database 2023

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) and the Therapeutic Innovation Networks (TINs), 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
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 - Principal 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**

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.

Simon Waddington is a Professor of Gene Therapy at UCL. He leads a team developing translational gene therapy for childhood inherited genetic diseases including neurodegenerative diseases, inherited epilepsy and metabolic diseases. He chairs the Cell and Gene Therapy Therapeutic Innovation Network of the UCL Translational Research Office.

With Professors Manju Kurian, Paul Gissen and Ahad Rahim, he has co-founded a company, "Bloomsbury Genetic Therapies". This has received £5 million of seed funding from Albion VC/ UCL Tech Fund. The company is progressing four gene therapy programmes to the clinic. They are gene therapy for Ornithine Transcarbamylase Deficiency, Infantile Neuronaxonal Dystrophy, Niemann Pick C and Dopamine Transporter Deficiency Syndrome. The company is now in the process of raising Series A funding. He and his team have been funded by the UK Medical Research Council Developmental Pathway Funding Scheme and LifeArc for numerous translational projects. They have licensed one of their projects to a gene therapy company with whom we have a £1.5 million sponsored research agreement in place. Collaborating with Prof Tristan McKay (Manchester Metropolitan University) he obtained a European Research Council starter grant of €1.5 million from the European Research Council to develop the means to quantify signalling pathways in diseased organs and tumours continually and non-invasively. Working alongside the teams of Professors Andy Baker (University of Edinburgh) and John McVey (University of Surrey) they elucidated one of the fundamental mechanisms by which adenovirus vectors transduce cells. He has several long-standing international collaborations. These include Drs Jerry Chan, Duke-NUS Graduate Medical School Singapore and Prof Patrick Arbuthnot, at the University of Witwatersrand, South Africa, where he holds an honorary chair.

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.

Professor Emma Morris is a clinician scientist, clinically trained in haematology, allogeneic haematopoietic stem cell transplantation (HSCT) and cellular therapies. Her current clinical practice is in HSCT, cellular and gene therapies for haematological malignancies and inherited immune deficiencies. She has developed and lead the national allogeneic HSCT centre for adults with primary immune deficiencies (PID) at the UCL Centre for Immunodeficiency and co-ordinate an international MDT to discuss the role of HSCT and gene therapy in adult PID patients.

Her basic science research group focuses on T cell immunology, inherited immunodeficiencies, gene therapy and gene editing. This research spans the entire translational pathway from animal models to in vitro experimental immunology to Phase I 'first time in man' clinical studies.

She was appointed Director of the UCL Division of Infection and Immunity in October 2019. She is also Director of the NIHR UCLH/UCL Biomedical Research Centre Infection, Immunopathology and Immunotherapeutics (III) Theme.

She is a member of the Wellcome Trust Career Development Awards Panel. She is patron of the charity Haematology Cancer Care (HCC) which raises money for UCLH.

She is a scientific founder of a UCL Spin-out, Quell Therapeutics Ltd, which is developing gene-engineered regulatory T cells for transplant tolerance and autoimmunity.

Professor Paul Gissen

Clinical Professor in Paediatric Metabolic Medicine and an Honorary Consultant at UCL Great Ormond Street Hospital for Children.

He obtained his medical degree from the University of Glasgow in 1995 and completed his Paediatrics training at Manchester, Sheffield and Birmingham Children's Hospitals specializing in inherited metabolic disorders.

During his PhD at Birmingham University, Professor Gissen identified genetic causes of several rare paediatric diseases and became interested in developing therapies for intracellular trafficking disorders such as Arthrogryposis, Renal Dysfunction and Cholestasis syndrome, Niemann Pick type C disease and Neuronal Ceroid Lipofuscinosis.

Professor Gissen is an NIHR Senior Investigator and Theme Leader of the Gene and Stem Cell Therapies theme at Great Ormond Street Hospital Biomedical Research Centre. He is also the academic co-founder of Bloomsbury Genetic Therapies and is the UK Chief Investigator for several clinical trials for Niemann Pick type C and Batten diseases.

Professor Bobby Gaspar

Honorary Professor of Paediatrics and Immunology, UCL Great Ormond Street Institute of Child Health.

Bobby is a world-renowned scientist and physician and accomplished strategic and organizational leader with more than 25 years of experience in medicine and biotechnology.

As one of Orchard's principal scientific founders, Bobby serves as chief executive officer of the company, and also sits on Orchard's board of directors. Bobby has been a pioneer in gene therapy and the evolution of hematopoietic stem cell (HSC) gene therapy technology – including some of the first studies in patients with severe combined immunodeficiency (SCID) – bringing it from some of the first studies in patients to potential regulatory approvals. His unparalleled expertise and deep relationships with key physicians and treatment centers around the world are integral to Orchard's efforts to identify patients with metachromatic leukodystrophy (MLD) and other diseases through targeted disease education, early diagnosis and comprehensive newborn screening.

Bobby is an honorary professor of pediatrics and immunology at the UCL Great Ormond Street Institute of Child Health and has led multiple clinical trials that have shown that gene therapy can successfully correct the genetic defect in immune deficiencies. He studied medicine and surgery at Kings College in London before completing his Ph.D. at the UCL Great Ormond Street Institute of Child Health.

Panel Discussion 1

Investment: Industry, VCs & Spinouts

Chair: Dr Anne Lane

CEO, UCL Business.

Anne has a PhD in medicine from UCL and an Executive MBA from Molson Business School, Montreal. After research at UCL and Harvard Medical School, Anne worked for RTP Pharma Inc in Montreal, out-licensing and preparing valuations of the company's portfolio for public listing. Anne joined UCL Ventures in 2000 and acted as consultant for the National Transfer Centre in the US. She is the CEO of UCLB, acts as director and interim CEO on several of UCLB's spinout companies and, is also a member of the Government Office for Technology Transfer (GOTT) Advisory Board.

Dr Richard Fagan

Director of BioPharm, UCL Business.

He is a cell and molecular biologist by training, gaining his PhD at McGill University, Canada. This was followed by a postdoctorate at the Imperial Cancer Research Fund, London, and then the National Institute for Medical Research. He left to join AstraZeneca as a team leader in the Department of Target Discovery, then worked for SanofiAventis as a senior team leader. In 2001 he left SanofiAventis and joined Impharmatica as Associate Director Target Discovery and worked very closely with the business development team. In 2006 he joined UCLB as Assistant Director BioPharm, becoming Director in 2007.

Dr Elisa Petris

Lead Partner, Syncona Investment Management Ltd.

She is a Director on the Board of Quell Therapeutics, Beacon Therapeutics and Forcefield Therapeutics and was previously on the Board of former portfolio companies Blue Earth Diagnostics and Neogene, and of current portfolio company Achilles Therapeutics. She was closely involved in the foundation of Quell, Blue Earth, Achilles and Beacon including their operational and strategic set-up.

Previously, she was a Senior Associate at Michel Dyens & Co. working on transactions covering the healthcare space, and a member of the Life Science team at L.E.K. Consulting based in London. While at L.E.K. she worked on projects for biotech, pharma and private equity clients. Elisa has a PhD in Molecular Biology from Imperial College and an MBA from London Business School.

Professor Emma Morris

See biography on page 6

Professor Bobby Gaspar

See biography on page 7

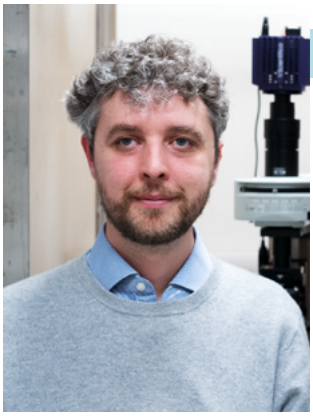
Session 2

Early-stage Research

Chair: Dr Rajvinder Karda

Associate Professor in Gene Therapy, EGA Institute for Women's Health, UCL.

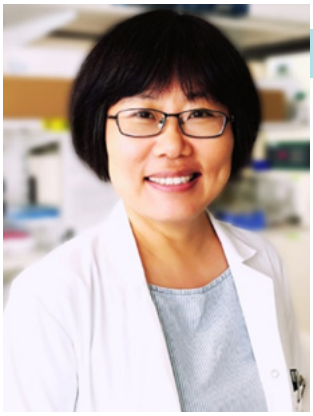
Dr Karda and her research team are focusing on developing novel gene therapy and RNA editing treatments for early onset, incurable genetic neurological and neurometabolic diseases. Dr Karda is a member of the scientific advisory committee for Dravet Syndrome Foundation, Spain and Cure DHDDS Charity, UK. She is also on the executive committee of British Gene and Cell Therapy Society. She has received funding from LifeArc and GOSHCC to develop novel genetic therapies for Dravet Syndrome and other inherited epilepsies.



Professor Gabriele Lignani

Professor of Translational Neuroscience, UCL.

He received his PhD in 2012 in Experimental Neuroscience from the University of Genoa and he did his first postdoc at the Italian Institute of Technology. Then he moved to UCL and was awarded a Marie-Curie individual fellowship to develop new CRISPR-based editing tools to treat epilepsy. In 2018 he started his own lab as Epilepsy Research UK Emerging Leader to further develop novel gene therapies for epilepsy. He then obtained an MRC New Investigator Award to develop new CRISPR-based technologies for Dravet Syndrome, recently an ERC Consolidator Grant, and several other discovery and translational grants. For his research in gene therapy for epilepsy he has recently been awarded the ILAE Harinarayan Young Scientist Award, the Michael Prize 2023 for the best scientific contribution to progress in the field of experimental epilepsy, the ILAE European Epilepsy Young Investigator Award 2024, and he has been elected Fellow of the Royal Society of Biology. The focus of his lab is to develop gene therapy and editing techniques for neurological disorders, to study the role of homeostatic plasticity in epilepsy and understand the basic epileptic mechanisms.



Dr Haiyan Zhou

Associate Professor, Genetics & Genomic Medicine Department, UCL.

Her research focuses on preclinical development of RNA-targeted novel therapies and translational biomedical research in rare genetic disorders. Dr Zhou serves as the deputy theme lead of the Accelerating Novel Therapy Theme in the NIHR Biomedical Research Centre at UCL Great Ormond Street Hospital, and leading the individualized RNA therapy Programme for paediatric rare diseases at GOSICH. She is the director of UCL Personalized Medicine and Novel Therapies MSc Programme, and holds the 2021 UK Harrington Rare Disease Scholar award. Dr Zhou leads the national programme on establishing a UK Platform of Nucleic Acid Therapy (UPNAT) for rare disease, a specialist rare disease node supported by the MRC and NIHR. Dr Zhou also leads in a number of large research projects on the preclinical development of RNA therapeutics in muscular dystrophies, neurodegenerative disorders (epilepsy and sensory neuropathy), respiratory disease and inborn errors of metabolism. The research topics in her group include the identification of novel therapeutic targets, design and validation of RNA-based therapeutic approaches (ASO, siRNA, microRNA, RNA/gene editing and mRNA), tissue/cell-specific delivery, and different model systems, functional assays and the eventual clinical translation.

Early Career Researcher Speakers

Dr Stephanie Efthymiou

Research Fellow, Department of Neuromuscular Diseases, UCL.

Dr Stephanie Efthymiou has graduated from Imperial College as a Biologist, and also spent a year in Lyon, France working on the pathogenesis of viruses. In 2014, she completed a Master's degree in Molecular Biology and the Pathology of Viruses at Imperial College, where she developed a deep interest in the molecular bases of disorders.

In 2015, she joined UCL as a member of the Wellcome Trust Strategic Award for Synaptopathies. She aimed to identify the genetic basis of rare paroxysmal disorders and helped to establish the SYNAPS Patient Group which has collected over 30,000 patient samples to date. In 2020, she received her PhD on neurogenetics, under the supervision of Prof Henry Houlden studying the genetic and functional basis of rare demyelinating neuropathies in children. She helped establish a genetic link for NFASC and NARS1 with early onset neurodevelopmental disorder.

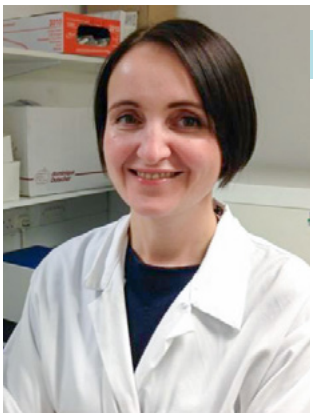
She continued with an ICGNMD Post-doctoral Research Fellowship under the supervision of Prof Michael Hanna. Her current research focus is studying genetic basis of developmental epileptic encephalopathies in patients from different ethnic groups. She is involved with the Rory Belle Foundation for NARS1 disease, helping build a patient community and driving treatment research and development.

Dr Annalucia Darbey

Research Fellow, Department of Neuromuscular Diseases, UCL.

Dr Annalucia Darbey joined the Fratta lab at UCL Queens Square Institute of Neurology as a Postdoctoral Research Fellow in 2021.

Her research focuses on using innovative and exciting technologies to develop tissue specific Gene Therapy solutions for Motor Neuron Diseases, particularly SBMA/Kennedy's Disease. She has been fortunate to receive a number of awards whilst at UCL including a Cell & Gene Therapy TIN Award and an AFM Telethon award – these have been crucial for my continuing development as an ECR. Prior to her work at UCL, she completed my PhD in Tissue Repair & Gene Therapy at The University of Edinburgh before moving to Newcastle, Australia for two years to investigate Gene Therapy for endocrine disorders as a Postdoctoral Research Associate.



Dr Amy McTague

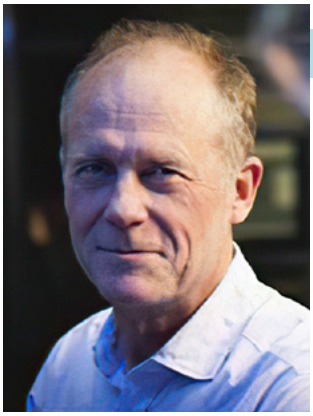
Clinical Consultant - Principal Research Fellow, Developmental Neurosciences Department, UCL.

Dr Amy McTague is a clinician scientist with a research group including a post-doctoral fellow and masters students at the Zayed Centre for Rare Disease Research in Children.

Her research vision is to move from precision diagnosis to precision treatment in childhood epilepsy. She is an honorary consultant paediatric neurologist at Great Ormond Street specialising in paediatric epilepsy, in particular early onset epilepsies. As a consultant in the Complex Epilepsy team she sees regional and national referrals and provide expert opinions on children with genetic epilepsies.She undertakes Epilepsy Genomics clinics and have established an Epilepsy Genomics MDT for phenotypic and variant classification. She is a co-investigator in trials of novel therapies for Dravet syndrome and in a Dravet syndrome natural history study.

Session 3

Early-stage Research



Chair: Professor Dimitri Michael Kullmann

Professor of Neurology Clinical & Experimental Epilepsy, UCL Queen Square Institute of Neurology.

His interests span the fundamental mechanisms of synaptic transmission, the computational properties of small neuronal circuits, and alterations in neuronal and circuit excitability in epilepsy and other neurological disorders. The core methods in his lab are in vitro electrophysiology and pharmacology, but they also apply confocal and two-photon laser scanning microscopy, computational simulations, molecular genetic methods, and heterologous expression of mutated ion channels. He collaborates with Michael Hanna, Henry Houlden, James Jepson, Shyam Krishnakumar, James Rothman, Kirill Volynski, Stephanie Schorge and many others to understand the mechanisms of inherited neurological diseases caused by mutations of ion channels (channelopathies). His laboratory has contributed to the discovery of silent synapses, glutamate spillover, presynaptic GABAA receptors in the cortex, human epilepsy caused by K+ and Ca2+ channel mutations, tonic inhibition in the hippocampus, and Hebbian and anti-Hebbian LTP in hippocampal interneurons.

Professor Amit Nathwani

Senior National Institute for Health Research (NIHR) Investigator and Professor of Haematology, UCL; Founder and CEO of NovalGen.

He received his medical degree from the University of Aberdeen and PhD in Molecular Biology from Open University, UK. After completing postgraduate training in Haematology, he embarked on a pivotal journey at St. Jude Children's Research Hospital in Memphis, Tennessee. It was here, under the guidance of the esteemed Dr Arthur Nienhuis, that Professor Nathwani developed a pioneering gene therapy approach for haemophilia B. This served as an important catalyst that changed the gene therapy landscape for monogenethic disorder. This was followed by development of technologies in his laboratory at UCL that led to successful gene therapy of haemophilia A. Professor Nathwani's research extends to a diverse range of translational gene transfer approaches focused on inherited disorders such as Fabry's and Gaucher's disease. These achievements culminated in the co-founding of Freeline Therapeutics in 2015, a UCL-spinout company aimed at advancing gene therapy solutions. In 2019, Professor Nathwani founded NovalGen to accelerate cutting-edge research from his academic laboratory into disruptive immunotherapy approaches for both cancer and non-malignant conditions.

Professor Nathwani is the recipient of the Ham-Wasserman Award, ESGCT Outstanding Achievement Award, Human Gene Therapy Award, and UCL Enterprise Award for his pioneering work in gene therapy and authored >100 peer-reviewed scientific papers.

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.

She leads an internationally recognised basic bench science and translational research team focussed on finding disease modifying therapies for Huntington's disease. She has led multiple first in human clinical trials as PI including the first successful phase 1/2b trial of an antisense oligonucleotide (NEJM 2019), and currently serves on several SABs advising industry on the development of potential gene targeting and nucleic acid therapies for HD. Sarah has over 380 peer-reviewed research publications and has received multiple awards for her research including, most recently, the 2022 MRC Millennium Medal for her outstanding achievements in medical research, and the 2023 Arvid Carlsson award from Lund University for her research in Huntington's disease mechanisms and therapeutics.

Panel Discussion 2

UCL's clinician's experience

Chair: Professor Dimitri Michael Kullmann

See biography on page 11

Professor Manju Kurian

Professor of Neurogenetics and NIHR Research Professor, UCL Great Ormond Street Institute of Child Health; Consultant Paediatric Neurologist at Great Ormond Street Hospital.

After graduating from Cambridge University, she trained in Paediatrics before subspecialising in Paediatric Neurology. At the end of her clinical training, she undertook a PhD (University of Birmingham) investigating the molecular genetic basis of childhood neurological disorders (2007-2011). She moved to UCL after her PhD, and is now an independent Principal Investigator at the Institute of Child Health.

She has been awarded a Wellcome Intermediate Fellowship (2012-2017), L'Oreal For Women in Science Award (2017), NIHR Professorship (2017-2022), ICNA Jon Stobo Award (2018) and the The Jules Thorn Award for Biomedical Research (2019-2024). Her grant income exceeds £10 million and she has >200 peer reviewed publications including works in Nature Genetics, Science, Science Translational Medicine and Lancet Neurology.

Her current research encompasses gene discovery for childhood neurological disorders, including early onset epilepsy, neurodegeneration and movement disorders. Her lab uses mainly cell models to investigate the underlying pathological basis of disease. She works closely with UCL Gene Therapy groups to develop novel therapeutic strategies for children with pharmacoresistant movement disorders. Her long term goal is to translate her research for patient benefit, through improved clinical diagnosis and precision medicine approaches.

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.

She graduated from Guy's, King's and St. Thomas' School of Medicine in 2001 and then trained in Paediatrics, subspecialising in Paediatric Immunology and Immunodeficiency. She undertook a Wellcome Trust funded PhD at UCL developing haematopoietic stem cell gene therapy, with continued NIHR and Wellcome Trust post-doctoral support to establish her own research group. She was appointed as a Consultant in Paediatric Immunology at Great Ormond Street Hospital in 2014.

Claire now works as a clinical academic leading an expanding number of gene therapy clinical trials at Great Ormond Street Hospital which treats patients with immune deficiencies, haematological and metabolic disorders. Her lab group is focused on developing novel therapies for immune system disorders using both gene therapy/gene editing and targeted small molecules. She has extensive experience of translating, leading, and delivering first in human clinical trials and the commercialisation pathway. As an attending physician

she oversees the clinical management of patients with immune deficiencies, including hematopoietic stem cell transplantation and maintains a strong interest in HLH disorders.

Claire is an internationally recognised expert in gene therapy and immunology, an elected board member of the European Society of Gene and Cell Therapy, Chair of the International Committee of the American Society of Gene and Cell Therapy and previously served two terms on the board of the British Society. She serves on the editorial board of several journals and grant review committees and holds an honorary position at Boston Children's Hospital/Dana Farber Cancer Institute and Harvard Medical School.

She is also the co-founder of the AGORA initiative (Access to Gene therapies fOr Rare disease) which has founding members across 6 European countries and brings together clinicians and scientist with direct experience of developing and delivering ex vivo gene therapies for rare diseases, aiming to facilitate access to effective gene therapies for treatment of patients with ultra-rare diseases.

Professor Amit Nathwani

See biography on page 11

Professor Sarah Tabrizi

See biography on page 12

Session 4

Cancer: CAR T-cell manufacture and therapies

Chair: Professor Daniel G. Bracewell

Professor of Bioprocess Analysis, Department of Biochemical Engineering, UCL.

Daniel has made major contributions to the fundamental understanding of the purification of biological products, including collaborations with Thailand, India and the USA. He has authored more than 100 peer reviewed journal articles in the area to date and currently supervises 15 doctoral and postdoctoral projects, many of these studies are in collaboration with industry. One such project was the basis from which the spinout Puridify a nanofibre absorption technology company now owned by Cytiva was created. He is academic lead for the UCL-Cytiva Centre of Excellence. He is currently developing cell free / enzymatic manufacturing technologies to enable personalised the cell and gene therapies.

Professor Qasim Rafiq

Professor of Bioprocess Engineering, Department of Biochemical Engineering, UCL.

Qasim has a focus on novel therapeutic modalities including Cell and Gene Therapies.

He is a multidisciplinary engineer working at the life science, engineering and commercial interfaces with a research focus on the bioprocessing, automation and biomanufacture of cell and gene-based therapies. He currently leads a research portfolio of > £7.5M as Principal Investigator and leads a dynamic interdisciplinary research group that collaborate internationally with high-calibre academic institutions, industry partners, and leading clinicians. He is also Programme Director of the new Manufacture and Commercialisation of Stem Cell and Gene Therapies MSc programme. Qasim is both a Chartered Engineer (CEng) and Chartered Scientist (CSci) and sits on multiple scientific and engineering committees including the IChemE Biochemical Engineering Subject Interest Group, British Standards Institute Biotechnology Committee and the BIA's Cell and Gene Therapy Advisory Committee.

Dr Claire Roddie

Consultant Haematologist at UCLH and Associate Professor in Haematology at UCL

She completed an Immunotherapy PhD at UCL with Karl Peggs and subsequently undertook a clinician scientist role with Martin Pule to develop the UCL CAR-T program.

Claire's current role involves pre-clinical development of novel cell therapy projects, GMP manufacture and clinical trial design. She is also responsible for the advanced therapies clinical service at UCLH.

Closing Remarks

Professor Geraint Rees

Vice-Provost (Research, Innovation & Global Engagement), UCL.

As UCL Vice-Provost, Geraint is responsible for providing vision and academic leadership for UCL's world-leading research, knowledge exchange and global engagement, and the functions, services and resources that support it.

Working closely with the UCL President & Provost, he will provide inspirational academic leadership for UCL's research, innovation and global engagement activity, and collaborate with other VP offices and Deans to ensure that research and innovation across the university continues to be of the highest quality and sustainability, delivering positive social impact.

Geraint has held a number of roles at UCL, including Dean of the Faculty of Life Sciences, Professor of Cognitive Neurology, Director of the Institute of Cognitive Neuroscience, UCL Pro-Provost (Academic Planning) and Pro-Vice-Provost (AI). He is a non-executive Director of UCL Business, one of the UK's most successful technology transfer companies, and was a Senior Scientific Advisor at DeepMind from 2018-2020.

Research Posters

1. **Sahil Patel**

   GRANPA: G-protein coupled Receptor Activated by Non-Prescription Agent- next generation chemogenetics with gene therapy potential.

2. **Braulio Carrillo Sanchez**

   Strategies for enhanced adeno-associated virus (AAV) vector formulations.

3. **Ruhina Maeshima**

   Development of in vitro transcribed RNA therapeutics for cystic fibrosis.

4. **Jenny Lange**

   A novel role for astrocytes in genetic epilepsies.

5. **Bessie Su**

   Identification of Itpk1 as a biomarker and a therapeutic target for breast cancer.

6. **Yiwen Li**

   Crafting Thermal Resilience: Rational Design of AAV Capsids.

7. **Anna Kowala**

   The role of collagen VI in satellite cell function.

8. **Amy Richardson**

   A novel KCC2-based gene therapy for the treatment of refractory epilepsy.

9. **Nishma Patel**

   Review: Cost-effectiveness of Chimeric Antigen Receptor (CAR) T-cell Therapy by Treatment Line.

10. **Robert Platten**

    Cost-efficient, Raman-enabled Bioreactor Monitoring.

11. **Yiman Li**

    Development of In Vitro Transcribed mRNA Therapeutics for Cystic Fibrosis.

12. **Shereen Nizari**

    Using non-invasive MRI to inform therapy by characterising fluid movement in the diseased brain.

13. **Sandeep Mangrati**

    pAAVing the way to high titres in stirred-tank bioreactors.

14. **Pedro Silva Couto**

    Scalable manufacturing of CAR-T cells using a non-viral approach and a transposon-transposase system.

15. **Laia Torres Masjoan**

    Optimizing the efficacy of antisense oligonucleotides for the treatment of Duchene Muscular Dystrophy.

16. **Geoffrey Howe**

    Stable nuclease secreting cells for dna impurity reduction in AAV manufacturing.

17. **Dara Annett**

    Novel Cyclosporine analogues enhance transduction efficiency in human stem cells via IFITM3 degradation.

18. **Enzo Giardina**

    Design of retinoid-responsive toxin gene systems for neuroblastoma treatment.

19. **Shunyi Ma**

    Establishment of a cellular model of SPTLC1-associated hereditary sensory neuropathy 1A to evaluate the efficiency of antisense oligonucleotides.

20. **Joseph Egan**

    Digital twin real-time predictions of optimal CAR T cell harvest times.

21. **Annarita Scardamaglia and Irem Karagoz**

    Toward Therapeutic Insights: Unveiling the Complexity of NARS1-Related Neurodevelopmental Disorder through Clinical and Molecular Investigations.

22. **Nivedhitha Swaminathan**

    Enhanced monitoring of T cell manufacturing with advanced process analytical technologies.

Symposium Organisers

UCL Translational Research Office (TRO) is an integral part of UCL's biomedical research powerhouse. As an expert team of applied scientists and business developers, we support our researchers in realising their translational ambition by offering strategic advice and funding support and bridging the gap with industry to help establish collaborations that drive the pipeline of therapeutic, device and diagnostic innovations.

UCL Translational Research Office

Learn more

Contact the TRO for partnerships:

Jane Kinghorn, Director: j.kinghorn@ucl.ac.uk

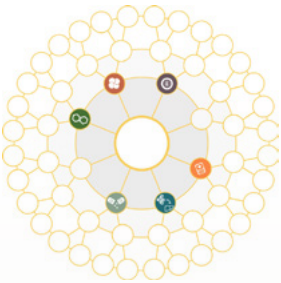
If you are a UCL researcher looking for translational research consultation or funding application support, please contact:

Pamela Tranter, Head of Translational Research Group: p.tranter@ucl.ac.uk

If you are an industry partner looking for collaboration opportunities with UCL, please contact:

Basma Jeelani, Head of Business & Innovation Group: b.jeelani@ucl.ac.uk

UCL Therapeutic Innovation Networks (TINs) are modality-focused agglomerations of UCL research expertise, centered around six of UCL's core strengths:



i. Small Molecules;
ii. Cell & Gene Therapies;
iii. Biologics;
iv. Re-purposing;
v. Devices & Diagnostics; and
vi. Regenerative Medicine.

The TINs were established by the UCL Translational Research Office (TRO), which brings together professionals from across academia and industry. Register with us to become a TINs member, get involved in our activities, and be the first to receive our news and event invitations.

Learn more

Join TINs and become part of the collaborative network:

1. Complete the form to become a TINs members and register your interest area.
2. Be ready to receive our newsletter, TINs updates and event invitations.

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UCL Business Ltd (UCLB), is a wholly owned subsidiary of UCL. Working with UCL's world-renowned researchers, faculties and associated hospitals we bring together exceptional ideas, innovations and industry to benefit society and the economy. UCLB's track record includes raising over £1.5 billion in investment for UCL spinouts.

Learn more

MOVING **SCIENCE** FORWARD

Biocair is a leading GDP logistics specialist dedicated to the life sciences sector. The company employs over 550 people and provides specialist logistics services to over 160 countries. Biocair's offices are located in the UK, France, Belgium, Germany, USA, South Africa, China, Singapore and India.

Learn more

Labcorp is a leading global life sciences company that provides vital information to help doctors, hospitals, pharmaceutical companies, researchers and patients make clear and confident decisions. Through our unparalleled diagnostics and drug development capabilities, we provide insights and accelerate innovations to improve health and improve lives.

Learn more

Bio-Techne empowers researchers in Life Science and Clinical Diagnostics by providing high-quality RUO and GMP reagents, instruments, custom manufacturing, and testing services. Our family of brands creates a unique portfolio of products and services: R&D Systems™ Novus Biologicals™ Tocris Bioscience™ ProteinSimple™ ACD™ ExosomeDx™ Asuragen™ Lunaphore™

Learn more

Cell & Gene

Lonza is a preferred global partner to the pharmaceutical, biotech and nutrition markets. We work to enable a healthier world by supporting our customers to deliver new and innovative medicines that help treat a wide range of diseases. We achieve this by combining technological insight with world-class manufacturing, scientific expertise and process excellence.

Learn more

GenScript, established in 2002, pioneers life science R&D and manufacturing globally. Catering to 200,000 scientists in 200+ countries, it offers diverse services like gene synthesis, peptide synthesis, protein expression, antibody development, and more, empowering breakthroughs in research and development.

Learn more

Thermo Fisher Scientific is the world leader in serving science. Together, we are making advancements that make a real difference. We do through product and service brands that include Thermo Scientific, Applied Biosystems, and Invitrogen. Our Mission is to enable our customers to make the world healthier, cleaner and safer.

Learn more

UCLB 30 years of commercialising technology

It's in our genes...

UCL's clinical innovation environment is grounded in a 'bench to bedside' approach with leading researchers working closely with UCL's associated hospitals including UCLH and Great Ormond Street Hospital.

Our stable of highly successful gene therapy spinouts, not least the five Nasdaq-listed companies, perfectly illustrate this translational synergy.

Dr Anne Lane, CEO, UCLB
"UCLB's Nasdaq-listed spinouts are changing lives and bringing new hope to cancer patients and people with genetic disorders across the globe. That's something we're incredibly proud of, and it keeps us focused on getting the next wave of great innovations out into the world where they can make a difference."

- UCLB's partnership with UCL's hospitals, researchers, clinical trial units and translational research office is one of the largest ATMP commercial pipelines in the world, with over 50 projects in the last three years alone.
- Specialisms in Rare Diseases, Ophthalmology, Haematology/ Oncology, Neurology and Immunology
- 12 successful spinouts in advanced therapeutics, five of which have received collective external investment of over £2bn.

Learn more about UCLB's impact.

Cocoon® Platform

The next step in cell therapy manufacturing

- Improved quality and efficiency
- Increased reliability and repeatability
- Reduced human error
- Less labor intensive = lower costs

Learn more

Lonza
Cell & Gene

Further Information

Location

- The event will take place in the Kennedy Lecture Theatre, UCL Institute of Child Health (ICH), 30 Guilford Street, London, WC1N 1EH. Please see the accessibility information here.
- Talks and presentations will take place in the ICH Kennedy Lecture Theatre
- Posters will be displayed in the ICH Winter Garden
- Lunch and refreshments will be served in the ICH Balcony and Winter Garden - Drinks reception will take place in the ICH Winter Garden

Connecting to Wifi

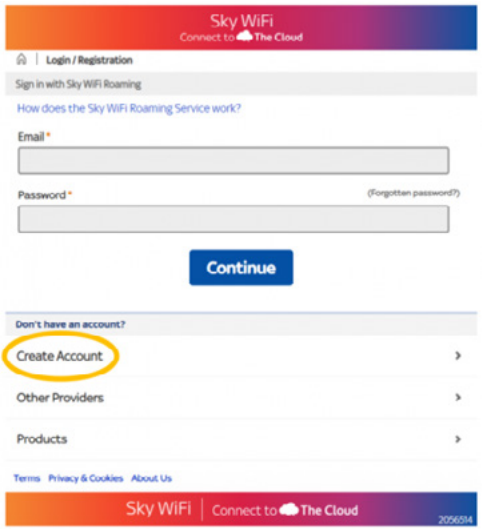
Before you start...

- You must be aware of and abide by the UCL Computing Regulations
- You must be aware of and abide by the JANET Acceptable Use Policy

Instructions

1. Select **UCLGuest** from your list of available Wi-Fi networks
2. Once connected, open a web browser and refresh your page
3. At the Welcome page (Fig.1) click **Go**

4. If you already have a The Cloud account, enter your email address and password and click **Continue**. If you do not have a The Cloud account, click **Create Account** to register (Fig.2)



5. After you have created a new account you will be connected to UCLGuest. You will also receive an email confirmation with your details.

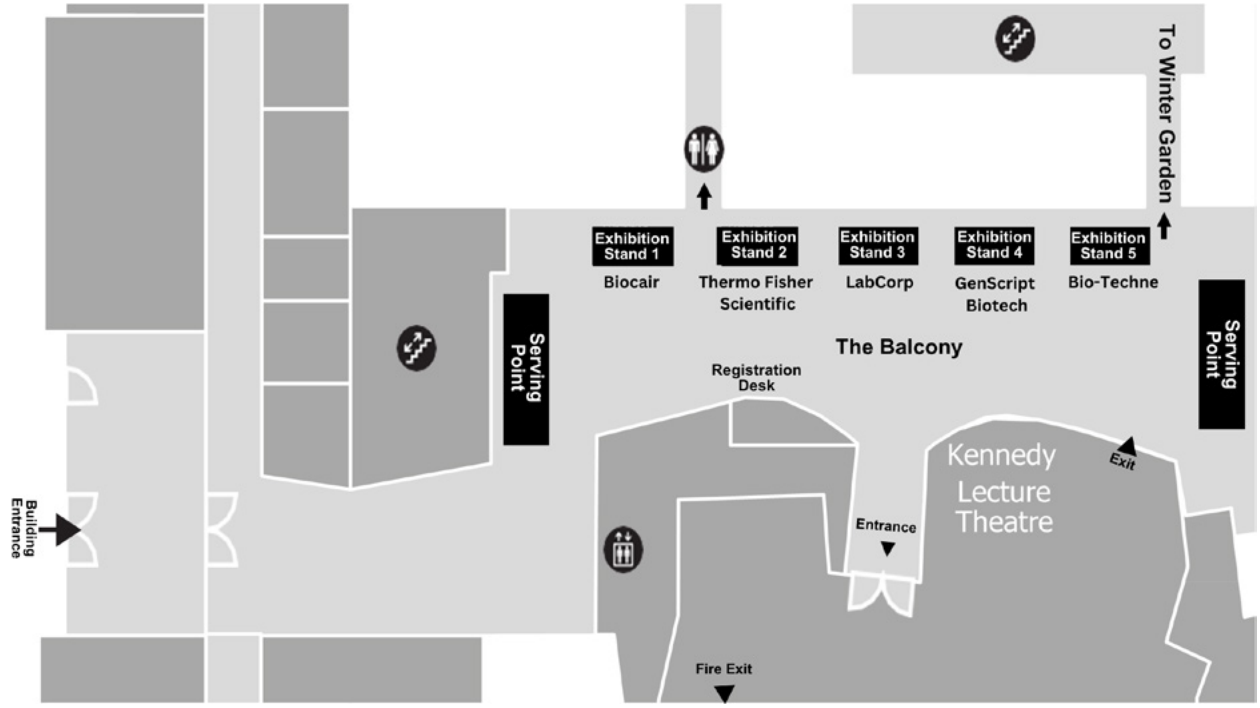
Having trouble connecting?

For further instructions please see Wi-Fi troubleshooting & known issues.

Venue layout and poster locations

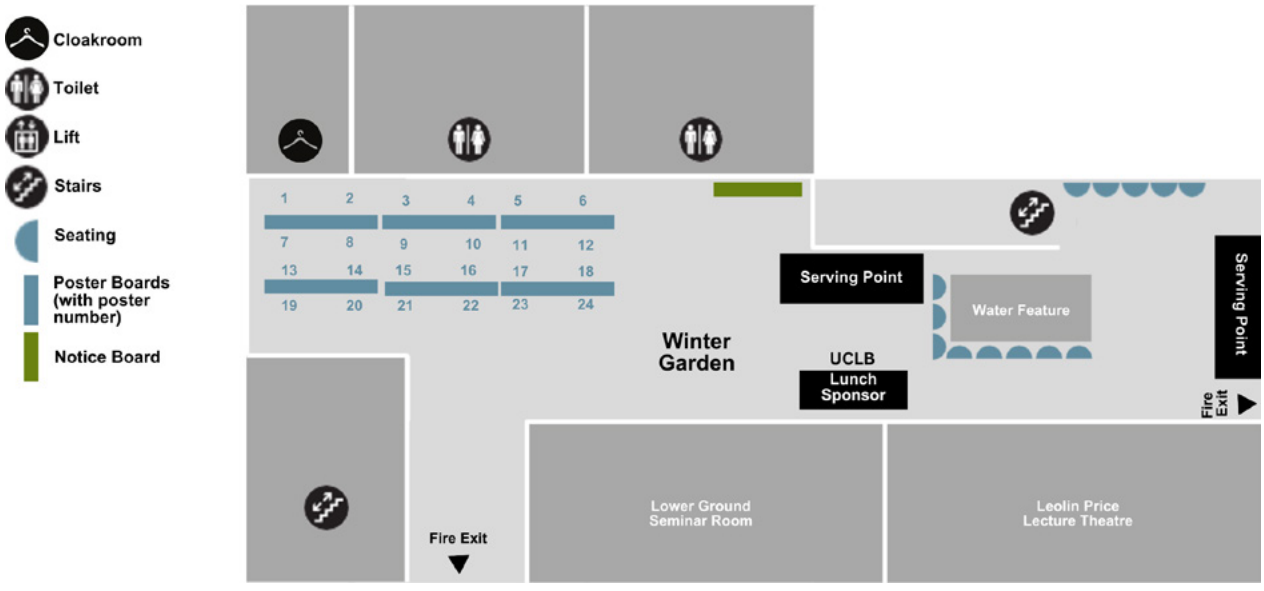
Kennedy Lecture Theatre & The Balcony

UCL Great Ormond Street Institute of Child Health



Winter Garden

UCL Great Ormond Street Institute of Child Health



UCL Advanced Therapies Symposium

Wednesday 24 April 2024
Registration 8.30
Seminar 9.00 – 17.30
Networking Reception 17.30 – 19.00

Kennedy Lecture Theatre,
UCL Great Ormond Street Institute of Child Health,
30 Guilford Street London WC1N 1EH

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