



UCL



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.

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Welcome

Dear Colleagues,

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

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.

excellence in advanced therapies across disciplines and the immense potential for translating research

- 1.
- 2.
- 3.
4. **Cancer: CAR T-cell manufacture and therapies.**

Panel discussions promise to bring thought-provoking insights on
as well as 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

Agenda

Wednesday 24 April 2024 | 09.00 – 17.30

Kennedy Lecture Theatre, UCL Great Ormond Street Institute of Child Health

Networks (TINs), which provides an invaluable opportunity for interaction with research and industry experts, fostering collaborations and connections within and beyond UCL.

8.30 – 09.00	Registration
09.00 – 09.10	Welcome and Introductions
	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
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 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
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](#)

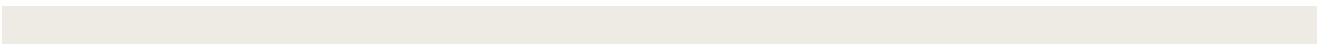
Dr Amy McTague, Clinical Consultant - Principal Research Fellow, Developmental Neurosciences Department, UCL
[A patient-derived neuronal epilepsy model towards novel therapy discovery](#)

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



Session 1

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.

gene-engineered regulatory T cells for transplant tolerance and autoimmunity.

Professor Paul Gissen

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

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

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.



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

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,

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 monogenetic disorder. This was followed by development of technologies in his laboratory at UCL that led to successful

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.

£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



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.

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

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

in human clinical trials and the commercialisation pathway. As an attending physician

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



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

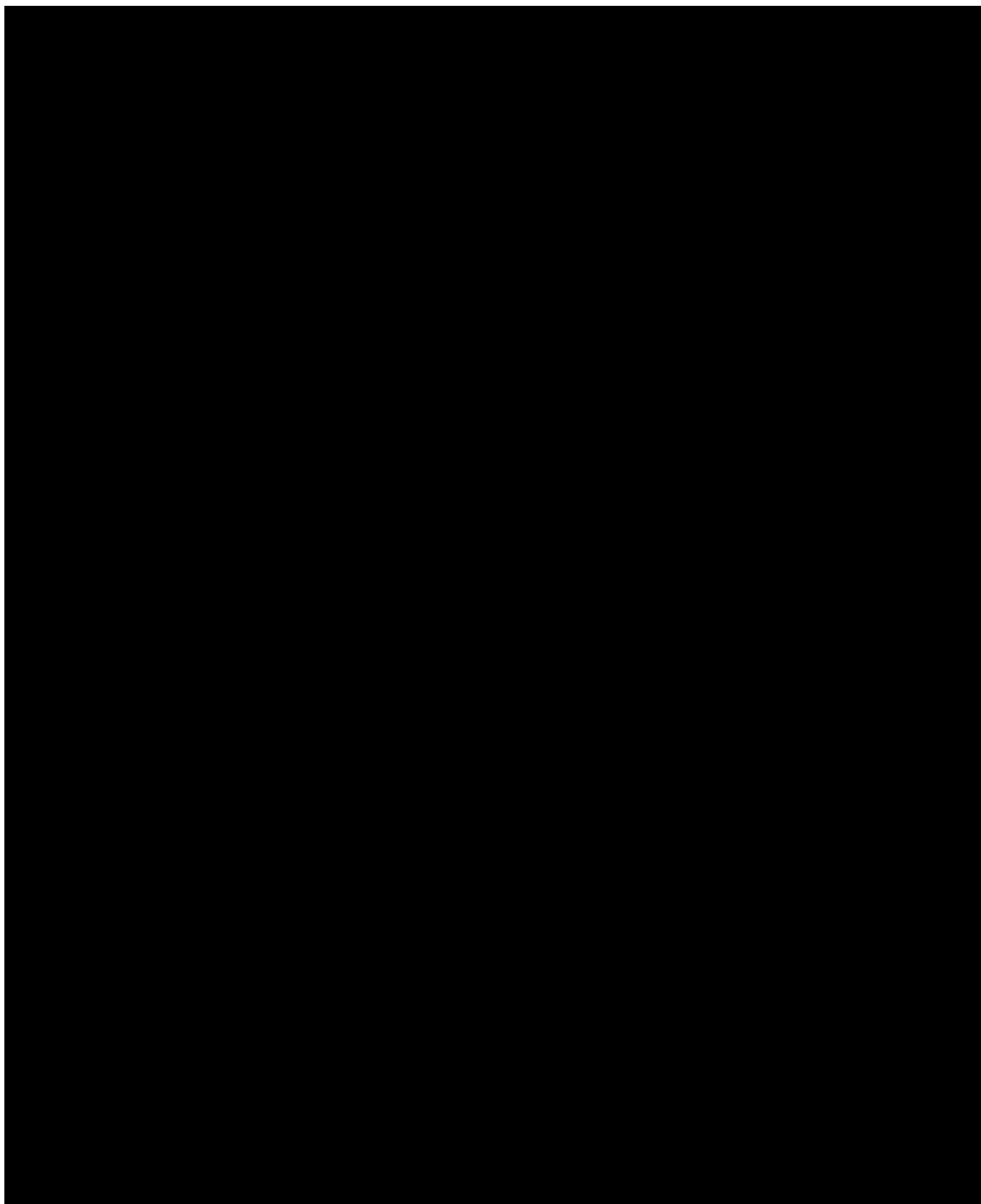
functions, services and resources that support it.

Working closely with the UCL President & Provost, he will provide inspirational academic

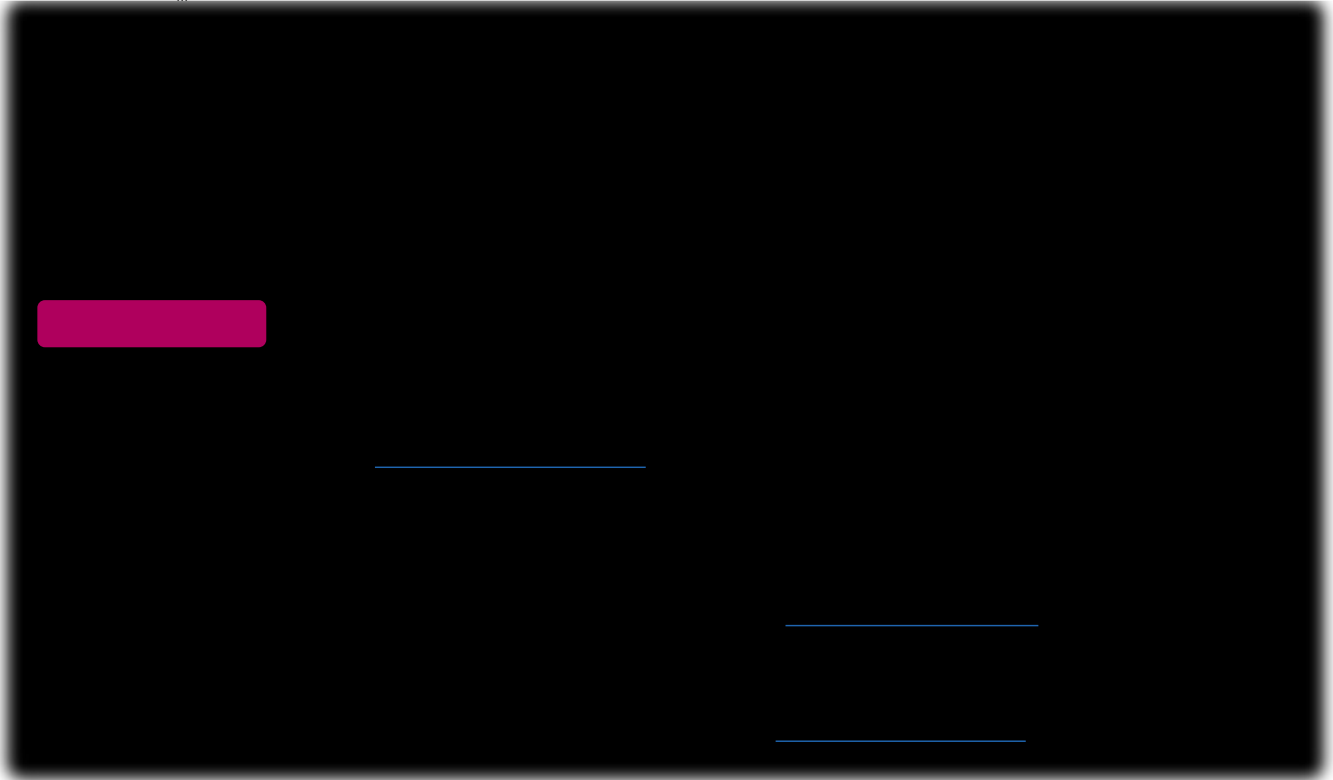
continues to be of the highest quality and sustainability, delivering positive social impact.

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

Research Posters



Symposium Organisers



Our Sponsors



UCL Business Ltd (UCLB)



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.

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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

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Before you start...

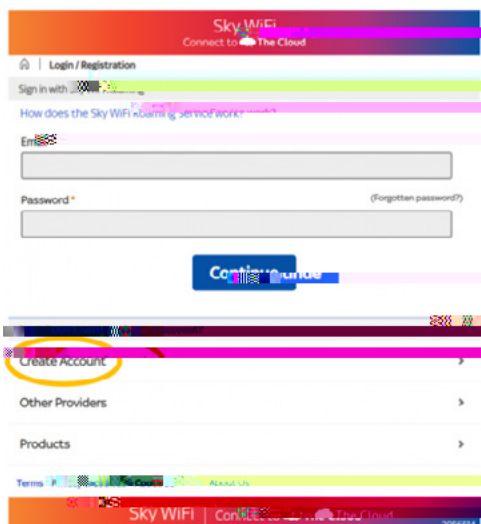
- You must be aware of and abide by the [UCL Computing Regulations](#)
- You must be aware of and abide by the [JANET Acceptable Use Policy](#)

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

Having trouble connecting?

For further instructions please see [Wi-Fi troubleshooting & known issues](#).

Venue layout and poster locations



