Experimental Medicine Training Initiative

PAEDIATRIC DRUG DEVELOPMENT

MONDAY, 8 OCTOBER 2018

ROBINSON COLLEGE, CRAUSAZ WORSWORTH BUILDING, GRANGE ROAD, CAMBRIDGE, CB3 9AN

UNIVERSITY OF CAMBRIDGE

Cambridge University Hospitals NHS Foundation Trust

Cambridge Biomedical Research Centre

NHS National Institute for Health Research

AstraZeneca

MedImmune

gsk GlaxoSmithKline

Learning Objectives

TO UNDERSTAND PAEDIATRIC DRUG DEVELOPMENT IN EXPERIMENTAL MEDICINE RESEARCH AND AS PART OF CLINICAL STUDIES

GAIN AN OVERVIEW OF DIFFERING DISEASE EPIDEMIOLOGY: PAEDIATRICS – ADULTS

REGULATORY CONSIDERATIONS
UNDERSTANDING CONDITIONS WHICH OCCUR IN PAEDIATRIC POPULATIONS & ADULT POPULATIONS

UNDERSTANDING THE DIFFICULTIES IN MANAGING A PAEDIATRIC PLAN FOR ACS INDICATION & ALTERNATIVE APPROACHES
INVESTIGATE THE UNIQUE ISSUES THAT DIFFERENTIATES PAEDIATRIC DRUG DEVELOPMENT FROM ADULT DRUG DEVELOPMENT.

INVESTIGATE AND DISCUSS THE CHALLENGES AND OPPORTUNITIES IN PAEDIATRIC TRIALS IN TYPE 2 DIABETES.

Resources

HTTP://WWW.EMI-TRAINING.ORG/

ROYAL COLLEGE OF PHYSICIANS CPD ACCREDITATION UNDER APPLICATION

SCHEDULE

FIRST SESSION		LEARNING OBJECTIVES AND INFORMATION
8.30-9.00am	**Registration and Refreshments**	
9.00-9.05	**Welcome** Dr Joseph Cheriyan/Dr Philip Ambery	Brief overview of the day.
9.05-9.35	**General overview of paediatric drug development** Professor Steve Cunningham, Professor of Paediatric Respiratory Medicine, Centre for Inflammation Research, University of Edinburgh	Gain an understanding of the challenges and processes around paediatric drug development.
9.35-10.05	**Paediatric Investigation Plans** Dr Phil Ambery Global Clinical Leader, Diabetes Astra Zeneca	Understanding the difficulties in managing a paediatric investigation plans.
10.05-11.15	**Pharmacokinetic and pharmacodynamic considerations in paediatric drug development** Marcella Petrone and Paul Baverel Principal Clinical Pharmacokineticist, MedImmune 30 minute talk interspersed with group exercises	How do we deal with nuances around PK/PD differences in children vs adults? Delegates will review and consider data to develop conclusions about the best approach to scaling dosage for children.
11.15-11.30	**Break**	
11.30-12.00	**Paediatric trials - a regulatory perspective** Dr Eleni Gaki Medical Assessor, Medicines and Healthcare Products Regulatory Agency (MHRA)	Learning about regulations around paediatric trials and understanding their application in trials.
12.00-12.30pm	**Regulatory considerations in paediatric drug development - an industry perspective** Ms Rhiannon Davies Global Regulatory Affairs Director at AstraZeneca Astra Zeneca	Learning about regulations around paediatric trials from an industry perspective and understanding their application in example trials.
12.30 – 1.30	**Lunch**	

SECOND SESSION		LEARNING OBJECTIVES AND INFORMATION
1.30-2.00	**Paediatric Vaccine Development** Dr Otavio Cintra Head of Scientific Affairs and Public Health Vaccines Global Medical Affairs GlaxoSmithKline	Investigate the unique issues that differentiates paediatric drug development from adult drug development.
2.00-3.00	**Difficulties in running a paediatric plan for Acute Coronary Syndrome (ACS) indication- Alternative approaches.** Dr Phil Ambery Global Clinical Leader, Diabetes Astra Zeneca	Explore alternative approaches in planning investigation in ACS indication in children
3.00-3.15	**Break**	
3.15-4.00	**Debate: -** **This house believes that paediatric trials are impossible to conduct in Type 2 diabetes.** **For:** Dr Phil Ambery **Against:** Dr David Dunger	Investigate and discuss the challenges and opportunities in paediatric trials in Type 2 diabetes.
4.00-5.00	**Closing comments, end and networking**	
5.30-6.30	**Drinks reception**	
6.30	**Dinner**	

SPEAKER BIOGRAPHIES IN ALPHABETICAL ORDER

Dr Philip Ambery
Senior Director, Clinical Biologics, Medimmune

Phil has considerable experience across all phases of clinical development in cardiovascular and metabolic diseases (CVMD) in the pharmaceutical industry. Having worked in medical affairs, late phase and early phase development for GSK from 2001-2014, he joined Medimmune where he is the lead member for the CVMD clinical group in Cambridge. He has a particular interest in incretins for the treatment of Type 2 diabetes and in Type 1 diabetes immune modulation. He is a specialist partner for the General Medical Council in academic or research certification, taking a particular interest in the licensing of doctors via the academic route, and still practises as an Acute Medicine Physician in Addenbrookes.

Dr Paul Baverel
Principal Clinical Pharmacokineticist, MedImmune

Paul's experience is in PK/PD analysis and pharmacology, with emphasis on early and late clinical development and translational biology. He joined MedImmune (Cambridge, UK) in August 2015 in the Translational Sciences department, providing clinical pharmacology and modelling and simulation support to the MedImmune pipeline of biologic molecules. Prior to MedImmune, Paul has worked at Pfizer and Eli Lilly. He holds a degree from the Pharmaceutical University of Paris (France) and completed his PhD in the field of modelling and simulation at Uppsala University (Sweden). His core responsibilities include using quantitative pharmacology in the support of projects in drug research and development, across several therapeutic areas, with the ultimate goal of setting the right dose in the right patient population.

Dr Joseph Cheriyan
Consultant Physician & Clinical Pharmacologist at Addenbrooke's Hospital, Associate Lecturer, University of Cambridge, Director of the Cardiovascular Trials Office, Vice Chair of the Cambridge Research Ethics Committee, EMI Training Lead

Joseph is an active clinical researcher with interests in cardiovascular medicine particularly vascular function and inflammation and is uniquely the only MHRA accredited Phase I/II clinical triallist on the Cambridge Biomedical Campus working on early phase experimental medicine studies since 2006. His post combines NHS research within a University Department, in close collaboration with GSK's only remaining in house Clinical Unit, where he is seconded as a Senior Clinical Pharmacologist.

Dr Otavio Augusto Leite Cintra MD, MsC, PhD
Head of Scientific Affairs and Public Health, GSK Global Medical Affairs, Vaccines, Belgium

Dr Cintra joined GSK in 2008 having had a long a prestigious career within paediatrics and infectious diseases. He graduated in 1989 from University of Sao Paulo School of Medicine at Ribeirao Preto and was board certified by the Brazilian Society of Paediatrics. His Masters came in 1998 and his PhD in 2002 focused upon RSV and other respiratory viruses. He has been a visiting professor to Baylor College of Medicine and Virginia School of Medicine. He was also Professor of paediatrics in University of Sao Paulo School of Medicine at Ribeirao Preto and has acted as principal investigator in various clinical trials. He has advised the Brazilian Ministry of Health and has had many peer reviewed articles, book chapters and abstracts as well as speaking at conferences. After 8 years working in GSK Vaccines Brazil and Latin America, he is presently the Head of Scientific Affairs and Public Health, GSK Vaccines Belgium.

Professor Steve Cunningham

Consultant in Paediatric Respiratory Medicine at the Children's hospital in Edinburgh. Vice Chair of the MHRA Paediatric Medicines Expert Advisory Group, Chair of the European Respiratory Society Clinical Research Collaboration.

With an emphasis on early life viral infection, Steve was Chair of the NICE Bronchiolitis guideline and Chief Investigator of BIDS (Bronchiolitis of Infancy Discharge Study) investigating oxygen saturation targets in acute vial bronchiolitis (BMJ Clinical Research Paper of the Year 2016). This work continues as PI for the IMI RESCEU study (a pan European RSV study of healthy infants) and within the RESPIRE NIHR Global Centre assessing respiratory disease in children in developing healthcare and as PI and Consultancy for new early phase therapeutic developments for RSV. Paediatric PI for the two clinical centre UK Cystic Fibrosis Consortium multidose gene therapy trial, and now focused on the delivery of small molecule therapies in very young children with cystic fibrosis modifying the early disease course. In Asthma, a member of the Asthma UK Centre for Applied Research, assessing near fatal asthma and how best to prevent asthma deaths. In Rare Lung Disease, a core member of the ChILDEU FP7 funded project, which is now followed as Chair of the European Respiratory Society ChILD Clinical Research Collaboration, looking to enhance global collaboration and novel therapeutics for ChILD.

Ms Rhiannon Davies

Global Regulatory Affairs Director at AstraZeneca

Rhiannon has extensive global regulatory experience (over 20 years) across a broad range of therapy areas and the whole development lifecycle. She has successfully provided strategic leadership of global health authority interactions across major regions including USA, EU and Japan. She has provided effective leadership and execution of successful global regulatory submissions including scientific advice, paediatric plans, marketing and clinical trial authorisations.

In addition to leading many knowledge sharing initiatives within AZ she was also the Module Leader for the TOPRA MSc Regulatory Strategy module for many years.

Prior to joining AZ in 2001, Rhiannon worked in global regulatory roles in 3M Healthcare and Smith & Nephew, supporting a broad portfolio including the first CFC-free pMDIs and novel cell based wound healing products.

Professor David Dunger

Professor of Paediatrics, University of Cambridge

David Dunger is a paediatric clinical scientist with over 30 years experience in three key research areas.

Pathogenesis of type 1 diabetes and its complications: In 1986 he initiated the Oxford Regional Prospective Study of childhood diabetes (ORPS).

Perinatal origins of risk for obesity and type 2 diabetes: This work began with an important collaboration with the ALSPAC study team exploring genetic determinants of size at birth.

Experimental Medicine: David Dunger is an international expert on the growth hormone/insulin like growth factor 1 axis and its effects on metabolism. Using type 1 diabetic subjects and people with growth hormone deficiency as models to explore these relationships he has carried out a series of physiological studies contrasting effects of growth hormone and IGF-1 on glucose metabolism and insulin. These led to the first experimental studies of rhIGF-1 in type 1 diabetes and subsequently a clinical trial published in the Lancet.

Dr Eleni Gaki

Senior Medical Assessor, Paediatric unit, Special Populations Group, Medical Health Products Regulatory Agency (MHRA)

Dr Gaki is a paediatrician who joined the Paediatric Unit of the MHRA in 2012. Her work as a medical assessor relates to the implementation of the EU Paediatric Regulation.

It involves, among other responsibilities, the assessment of Paediatric Investigation Plans and completed paediatric studies, as well as providing scientific advice on paediatric drug development. Eleni's area of interest is paediatric neurology and inborn errors of metabolism.

Dr Marcella Petrone
Principal Clinical Pharmacokineticist, MedImmune

Marcella is a clinical and preclinical pharmacokineticist with a strong interest in clinical pharmacology. She received her Biological Sciences Master's Degree at the University of Padova, (Italy) and a Research Fellowship at the institute of Pharmacology at the University of Padova, (Italy). After joining GlaxoWellcome's Centre of Excellence in Neuroscience and Anti-infectives Drug Development, she worked on preclinical aspects of small molecules drug metabolism and pharmacokinetics. Afterwards, she moved to Clinical Pharmacokinetics Modelling and Simulation departments with GSK, and then at Aptuit, where she applied clinical pharmacology sciences across different therapeutic areas. Her experience includes commercial development and consultancy on clinical and preclinical pharmacology and translational medicine for small biotechs approaching the early stage of clinical development. She joined Medimmune in 2015, where she provides input in the design, implementation and analysis of early stage clinical trials for peptides and antibodies in the cardiovascular and metabolic disease area.

Professor Ian Wilkinson
Professor of Therapeutics, Director of Cambridge Clinical Trials' Unit, Director of the Office of Translational Research, University of Cambridge

Ian has a long track record in clinical pharmacology and arterial haemodynamics. His research interest is in clinical/experimental studies designed to understand the mechanisms underlying arteriosclerosis and cardiovascular disease, and to understand the importance of novel biomarkers of arterial function in risk prediction. He directs the Cambridge Clinical Trials' Unit and is also a director of the Office of Translational Research in Cambridge. He has considerable experience of translational research, and in forming academic collaborations with Industry.

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