JPAG
JOINT PHARMACEUTICAL ANALYSIS GROUP

Paediatric Medicines: New and innovative Development Approaches

Thursday 16th May 2024

Royal Society of Chemistry, London

This must-attend event for anyone involved in addressing issues and challenges associated with development, safety and efficacy of paediatric formulations.

Development of paediatric formulations is linked to many challenges related to safety, efficacy, acceptability and patient adherence. The latter is a major challenge often dismissed in studies that particularly focus on the safety and efficacy of medicines. In addition, adherence can vary depending on the formulation and route of administration e.g. oral, parenteral, inhalation, etc.

This symposium focuses on integrated approaches across academia and industry to address challenges in the development, safety and efficacy of paediatric formulations and includes these key themes:

- Safety of paediatric formulations
- Challenges in paediatric drug delivery and clinical trials
- Advances in development of age appropriate oral paediatric medicines
- Characterisation of paediatric formulations
- Analysis in complex matrixes
- Taste assessment of formulation: acceptability versus safety
- Regulatory aspects

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Paediatric Medicines: New and innovative Development Approaches

Thursday 16th May 2024, *Royal Society of Chemistry, London*

Purpose of meeting

The purpose of the meeting is to stay up to date on new and changing guidelines, regulatory requirements and emerging technologies and their implementation in the field of paediatric medicines.

Challenges

The paediatric population is diverse, ranging from birth to adolescents. This brings challenges in the development of medicines that are suitable for the whole age range and requires specific consideration of the suitability of the excipients used as well acceptability of the dosage form.

Opportunities

This symposium brings together experts to discuss issues related to development and characterisation of medicines for children. It provides an opportunity to share knowledge and ideas and to network with colleagues working in the same area of the industry.

Proposed outcomes

- Clarity regarding the rationale behind guidelines
- Hear from experts on what is actually happening during implementation and in practice
- Practical advice from experts in the field on how to address challenges and realisation of the potential that exists
- Opportunities to challenge and raise concerns
- Chance to understand from experts in the fields how concerns may be addressed
- Learn about emerging technologies and scientific advances in the industry

Register today at www.jpag.org/cd171

POSTERS

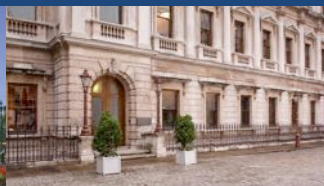
There will be a display of approved posters that delegates will be able to view and ask questions of the poster's author.

If you wish to present your own poster, please see the information on this on the JPAG website at www.jpag.org/info

EXHIBITORS

If you are a commercial company and would like to exhibit your products, see the JPAG website for details, at www.jpag.org/exhibitors



Programme

09.00 Registration and Refreshments

09.30 Welcome and introduction

09.40 Designing and delivering a successful study in paediatric medicines
Dan Hawcutt, Alder Hey Childrens' Hospital

10.10 Can Goldilocks get the right formulation to overcome the challenges of paediatric oral drug delivery?
Alice McCloskey, Liverpool John Moores University

10.40 Refreshments, Exhibition and Posters

11.00 Sensory Pharmaceutics™ and Paediatric Formulation Development
Catherine Tuleu, UCL School of Pharmacy

11.30 Predicting paediatric product performance using biopharmaceutics tools
Hannah Batchelor, Strathclyde University

12.00 Standardization of drug infusions in children
Sara Arenas-Lopez and Sian Gaze, Evelina London

12.30 Lunch, Exhibition and Posters

13.30 Analytical Testing Strategies for Pediatric OSDs
Beth Galella, Bristol Myers Squibb

14.00 Challenges of the Analytical testing that is required to support the handling instructions for paediatric formulations which are dosed with soft food
Jo Botterill, AZ analytical/food matrix/platform

14.30 Refreshments, Exhibition and Posters

14.50 The role of unlicensed medicines for paediatric patients
John Bardsley, Thistle Pharma Limited

15.20 UK and European Regulatory aspects of drug development in paediatrics
Shiva Ramroop, MHRA

15.50 Close

Programme subject to change. Copies of speakers presentations are available to registered delegates on the JPAG website, unless otherwise indicated.

Abstracts

Dr Sara Arenas-lopez - Paediatric Critical Care, Evelina London

Continuous, intravenous (IV) drug infusions are common in neonatal and paediatric critical care, and are typically used for life-sustaining medicines such as inotropes, analgesics, muscle relaxants, etc. Routine practice in these units is to withdraw an individualized dose of the required drug (calculated by weight) to a syringe of the appropriate size and then transfer to a second syringe, diluting to a larger volume of for example 50mL with Glucose 5% or Sodium Chloride 0.9%. This process occurs often at the bedside and produces a constant relationship between rate of administration and drug dose (for example, 0.1 mL/h of a morphine infusion usually equates to 5 mcg/kg/min if 2.5 mg/kg body-weight is diluted to a final volume of 50 mL). This is thought to facilitate bedside dose adjustment, and also limits fluid volumes. Medication errors associated with this practice are potentially harmful, and are three times more likely to occur in paediatric and neonatal populations than adults. The National Patient Safety Alert NPSA20 (March 2007) in the U.K, recognized this complexity, and recommended the use of standardised IV solutions provided in ready-to-use forms. This was also the outcome of the recent HSIB Report Investigation: Procurement, usability and adoption of 'smart' infusion pumps I2019/009 (2020)

Over the past 15 years significant work has been conducted nationally and internationally with well documented evidence suggesting that the use of standard concentrations of infusions is a significant improvement in safety and processes for transitioning from bespoke per weight to standard practices.

Several countries are now in the process of looking at standardisation of IV Medication for neonates and children including the U.K. through the Neonatal and Paediatric Pharmacy Group and the Royal College of Paediatrics Standard Infusions framework and the FDA via the American Society of Hospital Pharmacists national project "Standardise4Safety"

During our session the basis of the work informing the U.K. RCPCH framework will be described as well as the current national and international initiatives that aim to getting the product manufacturing and infusion medical devices aligning.

Dr Hannah Batchelor - Strathclyde Institute of Pharmacy and Biomedical Sciences

Regulations mandate age-appropriate products for paediatric populations however, access to clinical testing in these populations is limited. Therefore there is a need to use predictive physical or in silico models to predict product performance. This talk will provide an overview of the most suitable tools available and highlight the advantages of their use with some examples. Specifically use of advanced dissolution apparatus and PBPK models will be covered.

Joanne Botterill - Senior Scientist, AstraZeneca

Paediatric formulations for complex enabled formulations where solution and suspensions are not viable formulation options, often require additional development for children and infants (6 months to 7 years) who are unable to swallow capsules and tablets. Age-appropriate solid formulation administration often require dosing aids to enable the young children to take their medications. In particular, doses are often delivered by mixing with soft food.

The impact on product performance and stability of co-dosing with food and any hold times for the medicine in soft food prior to dosing is required to be understood to ensure safety and efficacy. Currently, there is no standardised way of performing this assessment. The presentation will discuss the analytical challenges of the testing required to support the handling instructions for dosing, and considerations for globally accepted understanding of demonstrating suitable foods for administration.

Elizabeth Galella - Bristol Myers Squibb

Due to the unique nature of pediatric patients, it is important to consider dose flexibility and acceptability attributes throughout the development of pediatric medicines. Dosage forms such as mini tablets and oral granules are formulation types that offer such benefits. Mini tablets, for example, may be packaged

in a variety of numbers and configurations in sachets, stick packs and/or bottles, with as little as one mini tablet or as many as hundreds packaged per dose. While these pediatric dosage forms must meet the same analytical testing criteria as standard adult dosage forms, there exists an opportunity to implement a more simplified approach to stability and release testing, while ensuring product quality.

Enhancing acceptability and compliance for pediatric formulations, on the other hand, often involves product manipulation which requires additional analytical assessment and may present challenges. In particular, dosing medication with food or liquid necessitates a vehicle selection process through compatibility and in-use stability assessments, as well as analytical method modification. This experimental work is critical for development of the dose preparation procedure and the instructions for use.

Sian Gaze - Medicine and Neonatology, Evelina London

Continuous, intravenous (IV) drug infusions are common in neonatal and paediatric critical care, and are typically used for life-sustaining medicines such as inotropes, analgesics, muscle relaxants, etc. Routine practice in these units is to withdraw an individualized dose of the required drug (calculated by weight) to a syringe of the appropriate size and then transfer to a second syringe, diluting to a larger volume of for example 50mL with Glucose 5% or Sodium Chloride 0.9%. This process occurs often at the bedside and produces a constant relationship between rate of administration and drug dose (for example, 0.1 mL/h of a morphine infusion usually equates to 5 mcg/kg/min if 2.5 mg/kg body-weight is diluted to a final volume of 50 mL). This is thought to facilitate bedside dose adjustment, and also limits fluid volumes. Medication errors associated with this practice are potentially harmful, and are three times more likely to occur in paediatric and neonatal populations than adults. The National Patient Safety Alert NPSA20 (March 2007) in the U.K, recognized this complexity, and recommended the use of standardised IV solutions provided in ready-to-use forms. This was also the outcome of the recent HSIB Report Investigation: Procurement, usability and adoption of ‘smart’ infusion pumps I2019/009 (2020)

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Dr Dan Hawcutt - Alder Hey Childrens Hospital

There are practical ways in which clinical trials, while continuing to adhere to the necessary regulatory standards, can be improved when they are involving children. This talk, from paediatric clinical pharmacologist with over 10 years paediatric clinical trial experience, and who is director of an early phase trials unit for children, will look at the study designs that help delivery successful studies, as well as key pitfalls seen in less successful studies. This will range from key overarching design considerations, to practical details that help recruit and retain participants and families.

Dr Alice McCloskey - Liverpool John Moores University

There is increasing acknowledgement that the one size fits all approach to medication use is not appropriate. This is particularly true for niche populations such as pediatrics. Children have very specific medication requirements based on their ever-changing physiology, need for regular review and calculation of doses, ability to take different formulations (either using devices correctly/ with assistance/

palatability/ ability to swallow solid oral dosage forms). As such the personalized approach seems attractive for those involved in the design and prescribing of medicines for children.
Our team and collaborators are exploring 3D-priniting as a strategy to develop child-appropriate solid oral medication. There are several examples highlighting the potential of 3D-printing to overcome the challenges faced in pediatric drug design with it offering a more tailored end-product that meets the patient's needs. To date, we have produced and analyzed polymer-based 3D printed gummy bears both with and without active pharmacuetical ingredients. We have also sought the views of parents/carers and children about these as potential medicines for children. This presentation will capture the key findings of our work thus far and the future directions of our research team.

Dr Shiva Ramroop - Team lead for Paediatric Unit HQA, MHRA

Drug and formulation development continue to be significant issues for children. The presentation provides an overview of the regulatory aspects of paediatric submissions in the UK and EU and some regulatory issues regarding development of the appropriate formulation in children.

Dr Catherine Tuleu - University College London

With examples and case studies, this presentation will provide an insight into the role of Sensory PharmaceuticsTM, integrating pharmaceutical sensory evaluation (preclinical, clinical) and formulation, dosage form design and administration factors research to ensure medicines are purposefully acceptable for children.

Speaker biographical details

Dr Sara Arenas-lopez - Paediatric Critical Care, Evelina London
Sara is a Consultant Pharmacist in Paediatric Critical Care.
She graduated as a pharmacist in 1995 from Universidad Complutense de Madrid, Spain. She was awarded 2 grants, to work in Bolivia (Universidad San Andres de La Paz) and The Netherlands to perform research in pharmaceutical technology at Utrecht University. Since 1997, she trained as a hospital pharmacist at the William Harvey and Great Ormond Street Hospitals. In 1999 and 2001, she carried out a collaborative project with the Zimbabwean Ministry of Health to help developing paediatric pharmacy services and, along with a project in paediatric intensive care in 2002, gained a Masters of Science Degree in Clinical Pharmacy from Brighton University.

For the past 24 years she worked at the Evelina London Children's Hospital, (Guy's & St Thomas's NHS Foundation Trust, London, United Kingdom) as a Paediatric Pharmacist. During this time she was seconded to the European Medicines Agency to work as a Scientific Paediatric coordinator for the Paediatric Committee evaluating Paediatric Investigational Plans (PIP's) and coordinating the Paediatric Formulation Working Group. She returned to the hospital as a Consultant Pharmacist for Paediatric Critical Care in May 2010 and completed a PhD in 2017 (International Degree University of Granada-Spain) primarily on the standardisation approach for paediatric IV medication Infusions and accuracy of enteral medical devices used in paediatric practice.

She collaborates as independent external expert for the European Medicines Agency and is a Honorary Senior Lecturer for UCL London School of Pharmacy, King's College London University and Tor Vergata University-Rome with extensive experience teaching nationally and internationally to medical, nursing and pharmacy professionals.

Dr Hannah Batchelor - Strathclyde Institute of Pharmacy and Biomedical Sciences
Professor Hannah Batchelor is a pharmaceutical scientist who has worked in academia, the NHS and within pharmaceutical industry. She is currently based at the Strathclyde Institute of Pharmacy and Biomedical Science, University of Strathclyde in Glasgow. She works on the design and manipulation of medicines to create age appropriate drug formulations to maximise clinical efficacy in paediatric patients. Her research interests lie in the optimisation of drug formulations to maximise their biopharmaceutical performance and acceptability to children. Her research is informed by the views of children, young people and parents to ensure that the patients are at the centre of new developments.
Hannah is the current chair of the Academy of Pharmaceutical Sciences in the UK. She is still passionate about pharmaceutical sciences and is motivated to inspire the next generation of scientists.

Joanne Botterill - Senior Scientist, AstraZeneca
Jo Botterill has over 25 years experience working in the analytical development department at AstraZeneca. She has worked on a variety of different dosage forms over her career, including pMDI's, Nasal sprays, solutions for infusion, tablets and capsules, and is currently the lead analyst supporting the development of two paediatric age-appropriate formulations.

Elizabeth Galella - Bristol Myers Squibb
Elizabeth Galella is currently a Senior Principal Scientist at Bristol Myers Squibb. Since joining BMS in 1997, Elizabeth's experience has been around analytical development and project management of small molecule drug products. Her role within the Oral Product Development department has also provided her opportunities to broaden her knowledge in the area of pediatric product development. Elizabeth is a member of the International Consortium for Innovation and Quality and co-led the IQ Pediatric Working Group through efforts to further the development of pediatric medicines.

Elizabeth Galella received her Master's degree in Chemistry from Rutgers University in 2001 and her B.S. degree in Chemistry from Douglass College in 1997.

Sian Gaze - Medicine and Neonatology, Evelina London
Sian has specialised in paediatrics for over 14 years, working at Evelina London Children's Hospital.
She completed her MSc at University College London in 2014 and became an independent prescriber in 2017.
In 2022, Sian became the Lead Pharmacist for Medicines and Neonatology at the Evelina and joined the Neonatal and Paediatric Pharmacy Group (NPPG) committee. She chairs the NPPG's NICU special interest group.
From 2014-22, Sian was employed at the Evelina as their Highly Specialist Neonatal Pharmacist, providing care to a 52-cot tertiary neonatal unit.
Sian also worked 1-day a week at Demelza Children's Hospice from 2018-2022 in a palliative care role.
She is passionate about education and training and is an Honorary Senior Clinical Lecturer at King's College London University.

Dr Dan Hawcutt - Alder Hey Childrens Hospital
Dr Hawcutt is a Reader in paediatric pharmacology at the University of Liverpool, and honorary consultant in paediatrics at Alder Hey Children's Hospital. He is Director of Research at Alder Hey Children's Hospital, and Director of the NIHR Alder Hey Clinical Research Facility (CRF). Dr Hawcutt is also chair of the Royal College of Paediatrics and Child Health (RCPCH) / Neonatal and Paediatric Pharmacists Group (NPPG) joint standing committee on medicines, and a member of the Medicines and Healthcare Regulatory Agency Pharmacovigilance Expert Advisory Committees on both Pharmacovigilance and Paediatric Medicines. In addition, he is chair of the RCPCH training committee for Paediatric Clinical Pharmacology.

Dr Alice McCloskey - Liverpool John Moores University
Dr McCloskey is a Reader of Pharmacy and Pharmacy programme leader at Liverpool John Moores University. She is a UK registered pharmacist with a range of experience working across the three main sectors of pharmacy- clinical practice, academia and industry. Upon successful completion of her pre-registration training Dr McCloskey returned to Queen's University Belfast and completed a PhD in pharmaceutics where she developed 'Self-assembled Peptide Nanomaterials for the Prevention of Biomaterial Infection'. This was followed by an industry-academia collaborative post-doc between the Royal College of Surgeons in Ireland (RCSI, Dublin) and the Galway-based nebuliser company Aerogen, a global leader in aerosol delivery. This work was also linked with a national patient focused medical device research centre, CURAM based at The National University of Ireland, Galway. Dr McCloskey is a Fellow of the Royal Pharmaceutical Society and Fellow of HEA. Links are maintained with clinical pharmacy through regular days in practice both in hospital and community settings. Her current research interests include self-assembling nanostructures for biomedical applications, medication use and design of medicines for patients at the extremes of age, and preparation of pharmacy students for real-life practice.

Dr Shiva Ramroop - Team lead for Paediatric Unit HQA, MHRA
Currently Senior Medical Assessor at the MHRA.
Team lead for the Paediatric Unit which includes review of paediatric investigation plans, scientific and regulatory advice on paediatric development.
Previously worked in the NHS for 16 years, with a background in Paediatrics and Infectious Diseases.

Dr Catherine Tuleu - University College London
Catherine TULEU, Docteur en Pharmacie, PhD, is Professor in Paediatric Pharmaceutics at UCL School of Pharmacy, UK. Her research, inherently translational, ranges from formulation, methodology development to clinical implementation, integrating the following themes: children centric excipient research; repurposing by reformulating for better medicines for children; development of innovative age appropriate dosage forms (especially for under 5s); administration issues and devices and sensory pharmaceuticsTM (dosage form acceptability and in vitro/in vivo taste assessment).
Her spin out company senCeuTics Ltd. specializes in pharmaceutical sensory evaluation and offers a full spectrum of preclinical, clinical and paediatric formulation services under one roof.
She is the founder and chairperson of the European Paediatric Formulation Initiative (EuPFI), a consortium working in a pre-competitive way on paediatric drug formulations.

VENUE: Royal Society of Chemistry, Burlington House, Piccadilly, London W1J 0BA

FEES: £195 Members
£295 Non-members
£45 Students and Concessions
See JPAG website for more details

TRAVEL: The RSC is very conveniently located in central London, with Piccadilly Circus and Green Park Underground stations just a few minutes' walk away. The nearest train stations are Charing Cross (10 minutes' walk), Waterloo, Victoria and Kings Cross/St Pancras (10 minutes by taxi).

ACCOMMODATION:
See JPAG website for details

OTHER: For other details, see JPAG website at www.jpag.org/info

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The RSC at Burlington House, Piccadilly

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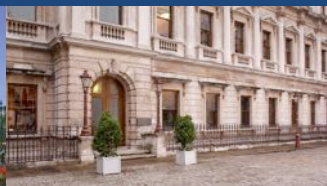

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