The University of Maryland Center for Excellence in Regulatory Science and Innovation, in Collaboration with the European Paediatric Formulation Initiative (EuPFI) and the IQ Consortium, Presents:

Challenges and Strategies to Facilitate Formulation Development of Pediatric Drug Products

Financial Assistance Provided by AbbVie, Bristol-Myers Squibb, Lilly, and Takeda

College Park Marriott Hotel and Conference Center

June 8-9, 2016

Hyattsville, MD

Conference Agenda

Overview Agenda

Day 1: June 8, 2016

Time	Activity
8:00-8:05 a.m.	Welcome and Workshop Goals
8:05-8:20 a.m.	Plenary Opening
8:20-10:30 a.m.	Session 1: Age Appropriate Formulation – General Considerations **Session Chairs:** Anne Zajicek (NIH), Hari Sachs (FDA), Rob Ju (AbbVie), and Brian Aylward (Irish Medicines Board)
10:30-11:25 a.m.	Acceptability Assessment Opening
11:25-3:00 p.m.	Session 2: Acceptability Assessment of Pediatric Formulations – Swallowability **Session Chairs:** Arzu Selen (FDA), Fang Liu (University of Hertforshire), and Siri Wang (Norwegian Medicines Agency)
3:00-5:15 p.m.	Session 3: Acceptability Assessment of Pediatric Formulations – Palatability **Session Chairs:** Bob Ternik (Eli Lilly and Company) and Siri Wang (Norwegian Medicines Agency)
5:15-5:30 p.m.	Wrap-Up for Day 1

Day 2: June 9, 2016

Time	Activity
8:00-12:00 p.m.	Session 4: Food Effects in Pediatric Medicines Development for Products Co-administered with Food **Session Chairs:** Andrew Mulberg (FDA), Hannah Batchelor (University of Birmingham), and Ann Marie Kaukonen (Finnish Medicines Agency) Session 5: Safety Qualification of Excipients **Session Chairs:** Darren Fegley (FDA), Lorrene Buckley (Eli Lilly and Company), Jacqueline Carleer (Belgian Federal Agency for Medicines), and Gerri R. Baer (FDA)

1:00-4:00 p.m.	**Session 6: Biopharmaceutics and Clinical Pharmacology** **Session Chairs:** James Polli (University of Maryland School of Pharmacy), Jian Wang (FDA), Brian Aylward (Irish Medicines Board), and Tycho Heimback (Novartis)
4:00-4:30 p.m.	**Wrap-Up for Day 2**

Detailed Agenda

Day 1: June 8, 2016

Time	Activity
8:00-8:05 a.m.	**Welcome and Workshop Goals**
8:05-8:20 a.m.	**Pediatric Global Regulatory Overview: Status, Challenges, and Opportunities with Focus on Pediatric Formulation Development** **Lynne Yao, MD** Director, Division of Pediatric and Maternal Health Office of Drug Evaluation IV Food and Drug Administration
	Sessions 1-3: Age Appropriate Formulation and Acceptability Assessment Regulations in both the EU and US require that pediatric drug products be appropriate for use in the target population. There has been significant discussion on demonstrating acceptability of dosage forms for pediatric patients. Formulations can be optimized based on pharmacokinetics, taste and overall acceptability measures. Given the high heterogeneity of the global pediatric population and the wide variety of existing and emerging formulation options, attributes of product acceptability are complex. The session purpose is to discuss patient acceptability and clinical performance from a global perspective, and will focus on palatability and swallowability of oral dose forms. These attributes will serve as examples around which a rational risk based approach for determining acceptability can be proposed, including: sources of data to demonstrate acceptability of palatability and swallowability; example methodology for use in assessing palatability and swallowability; and approaches to establishing criteria for acceptability.
	Session 1: Age Appropriate Formulation -- General Considerations **Session Chairs:** Anne Zajicek (NIH), Hari Sachs (FDA), Rob Ju (Abbvie), and Brian Aylward (Irish Medicines Board)

8:20-8:30 a.m.	**Gastrointestinal Physiology in Pediatrics: Implications for Pediatric Formulation Development** **Andrew Mulberg, MD, FAAP** Deputy Director Division of Gastroenterology and Inborn Errors Products Food and Drug Administration
8:30-8:40 a.m.	**Pediatric Formulation Development: Opportunities from an Industry Perspective** **Robert Ternik, PhD** Senior Research Advisor and Design Team Leader Eli Lilly and Company
8:40-8:50 a.m.	**EMA/PDCO Pediatric Formulation Working Group Experience** **Brian Aylward, MD** Clinical Assessor Health Products Regulatory Authority Irish Medicines Board
8:50-9:00 a.m.	**FDA Clinical Perspectives** **Erica Radden, MD** Pediatric Team Member Division of Pediatric and Maternal Health Office of Drug Evaluation IV Food and Drug Administration
9:00-9:10 a.m.	**EuPFI and Pediatric Formulation Development** **Catherine Tuleu, PhD** Reader in Pharmaceutics University College London School of Pharmacy
9:10-9:20 a.m.	**Pediatric Formulations Research: NIH Perspectives** **Anne Zajicek, MD, PharmD** Chief, Obstetric and Pediatric Pharmacology and Therapeutics Branch Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)
9:20-10:05 a.m.	**Panel Discussion** **Panelists:** Drs. Yao, Mulberg, Ternik, Aylward, Radden, Tuleu, and Zajicek
10:05-10:30 a.m.	**Break**
	Acceptability Assessment of Paediatric Formulations: Opening for Sessions 2 and 3
10:30-10:55 a.m.	**EMA Regulatory Perspectives** **Ann Marie Kaukonen, PhD**

Senior Researcher/Pharmaceutical Assessor
Finnish Medicines Agency

10:55-11:20 a.m.

FDA Product Performance/Chemistry Perspective

Arzu Selen, PhD
Associate Director, Scientific Development
Office of Testing and Research
Office of Pharmaceutical Quality
Center for Drug Evaluation and Research
Food and Drug Administration

11:20-11:25 a.m.

Sessions 2 and 3: Breakout Logistics and Goals

Session 2: Acceptability Assessment of Pediatric Formulations -- Swallowability

Session Chairs: Arzu Selen (FDA), Fang Liu (U of Hertfordshire), and Siri Wang (Norwegian Medicines Agency)

11:25-11:40 a.m.

Literature Review

Fang Liu, PhD, MSc, BSc
Senior Lecturer in Pharmaceutics and Drug Delivery
University of Hertfordshire

11:40-12:00 p.m.

Industry Perspective

David Tan Cheng Thiam
Associate Scientist
AbbVie

12:00-1:00 p.m.

Lunch

1:00-2:20 p.m.

Breakout Discussions

Case studies of swallowability questions will be provided. Participants will be broken up into smaller groups to discuss potential solutions.

2:20-2:35 p.m.

Break

2:35-3:00 p.m.

Summary Reports from Breakout Discussions

Session 3: Acceptability Assessment of Pediatric Formulations -- Palatability

Session Chairs: Bob Ternik (Lilly) and Siri Wang (Norwegian Medicines Agency)

3:00-3:15 p.m.

Literature Review

Yuet Mei Khong, PhD
Senior Scientist/Group Leader
AbbVie

3:15-3:30 p.m.

Pediatric Formulation Development: Industry Perspective on Palatability Challenges and Opportunities

Jeremy Bartlett, PhD

	Associate Research Fellow Pharmaceutical Sciences Drug Product Design Pfizer, Inc.
3:30-4:50 p.m.	**Breakout Discussions** Case studies of palatability questions will be provided. Participants will be broken up into smaller groups to discuss potential solutions.
4:50-5:15 p.m.	**Summary Reports from Breakout Discussions**
5:15-5:30 p.m.	**Wrap-Up for Day 1**

Day 2: June 9, 2016

Time	Activity
	NOTE: Sessions 4 and 5 will be held in parallel.
	Session 4: Food Effects in Pediatric Medicines Development for Products Co-administered with Food **Session Chairs:** Andrew Mulberg (FDA), Hannah Batchelor (University of Birmingham), and Ann Marie Kaukonen (Finnish Medicines Agency) The objectives of this session include: identify best practices/process flow (based on current information) that could be used to de-risk the formulation development strategy; and plan appropriate clinical studies and subsequent label claims for pediatric products that are co-administered with food. Gaps where more research or data is required will also be identified.
8:00-8:05 a.m.	**Introduction** **Session Chairs:** Andrew Mulberg (FDA), Hannah Batchelor (University of Birmingham), and Ann Marie Kaukonen (Finnish Medicines Agency)
8:05-8:25 a.m.	**In Vitro Tools to Risk Assess the Likelihood of a Food/Vehicle Effect in Pediatric Populations** **Sandra Klein, PhD** Professor of Pharmaceutical Technology University of Greifswald
8:25-8:50 a.m.	**Preclinical In Vivo, Clinical PK and PKPD Tools to Risk Assess Food/Vehicle Effects** **Barbara Davit, PhD** Executive Director Merck
8:50-11:30 a.m.	**Breakout Discussions**

Case studies of food/vehicle effect questions will be provided. Participants will be broken up into smaller groups to discuss potential solutions.

10:00-10:15 a.m.

Break

11:30-12:00 p.m.

Summary Reports from Breakout Discussions

Session 5: Safety Qualification of Excipients used in Pediatric Formulations

Session Chairs: Darren Fegley (FDA), Lorrene Buckley (Eli Lilly and Company), Jacqueline Carleer (Belgian Federal Agency for Medicines), and Gerri R. Baer (FDA)

The objective of this session is to identify how current regulatory guidance is interpreted by various stakeholders and to characterize approaches to qualify pediatric excipients. Experiences from both the nonclinical and clinical perspectives will be highlighted for possible paths forward towards more consistent, risk-based approaches.

8:00-8:15 a.m.

Overview of Issues

Darren Fegley, PhD
Pharmacologist/Toxicologist
Food and Drug Administration

Lorrene Buckley, PhD, DABT
Toxicologist
Eli Lilly and Company

Jacqueline Carleer, DVM
Non-clinical Assessor
Belgian Federal Agency for Medicines

Gerri R. Baer, MD
Medical Officer
Office of Pediatric Therapeutics
Food and Drug Administration

8:15-8:35 a.m.

Current Clinical Perspective on Risk Assessment

Mark Turner, PhD
Senior Lecturer in Neonatology
University of Liverpool

8:35-9:15 a.m.

Panel Discussion

Facilitators: Lorrene Buckley (Lilly) and Mark Turner (University of Liverpool)

Panalists: Darren Fegley (FDA), Brian Aylward (Irish Medicines Agency), Gerri Baer (FDA), Karen Thompson (Merck), Smita Salunke (UCL School of Pharmacy), and Jacqueline Carleer (Belgian Federal Agency for Medicines)

9:15-11:30 a.m.	**Breakout Discussions** Case studies of safety qualification of excipients questions will be provided. Participants will be broken up into smaller groups to discuss potential solutions.
10:00-10:15 a.m.	**Break**
11:30-12:00 p.m.	**Summary Reports from Breakout Discussions**
12:00-1:00 p.m.	**Lunch**
	Session 6: Biopharmaceutics and Clinical Pharmacology Considerations **Session Chairs:** Jian Wang (FDA), James Polli (University of Maryland School of Pharmacy), and Brian Aylward (Irish Medicines Board) The objectives of this session include: discuss considerations from a perspective of clinical pharmacology and biopharmaceutics; and identify the role of physiologically-based pharmacokinetic (PBPK) modeling and a pediatric BCS in drug development, class boundaries, and necessary research for a pediatric BCS. Topics include age range, BCS class boundaries, and frameworks for predicting drug absorption in paediatric patients.
1:00-1:15 p.m.	**Formulation-dependent Pediatric Physiologically-Based Pharmacokinetic (PPBPK) Modeling to Aid Drug Development** **Wen Lin, PhD** Senior Investigator Novartis
1:15-1:30 p.m.	**Biopharmaceutical Considerations in Pediatric Formulation Development** **Jack Cook, PhD** Clinical Pharmacology Leader Pfizer, Inc.
1:30-1:45 p.m.	**Proposed BCS for Pediatrics and Implication on Bioequivalence Assessment** **James Polli, PhD** Shangraw/Noxell Endowed Chair in Industrial Pharmacy and Pharmaceutics Department of Pharmaceutical Sciences University of Maryland School of Pharmacy
1:45-2:00 p.m.	**Clinical Pharmacology Considerations of Pediatric Formulation: Case Studies on Antiviral and Anti-infective Products** **Shirley Seo, PhD** Clinical Pharmacology Team Leader, Antiviral Products Food and Drug Administration

2:00-3:00 p.m.	**Breakout Discussions** Case studies of biopharmaceutics and clinical pharmacology questions will be provided. Participants will be broken up into smaller groups to discuss potential solutions.
3:00-3:10 p.m.	**Break**
3:10-4:00 p.m.	**Panel Discussion of Breakout Discussions** **Panelists:** Drs. Seo, Fang, Polli, Heimbach, Kim, Abernethy, Suarez, Cook, Green, Liu, Aylward, and Burckart
4:00-4:30 p.m.	**Wrap-Up for Workshop**

Transportation Information:

If you are staying at the **Greenbelt Marriott Hotel**, a shuttle service has been arranged to take you to and from the meeting on June 8 and 9.

- **Morning Shuttle Pick-Up Time**: Begin boarding at 7:00 a.m. at the Greenbelt Conference Center entrance in the REAR of the hotel. The shuttle departs promptly at 7:15 a.m.
- **Evening Shuttle Pick-Up Time**: Begin boarding at 5:45 p.m. outside of the Conference Center entrance alongside the UMUC garage entrance. The shuttle departs promptly at 6:00 p.m.