



# Public Workshop

## Pediatric Clinical Investigator Training

Pooks Hill Marriot (Bethesda, MD)  
September 22, 2014

### AGENDA (DRAFT)

## “Pediatric Clinical Investigator Training”

**8:00-8:10 am**

### Welcome and Introduction

Dianne Murphy, M.D., F.A.A.P., Director, Office of Pediatric Therapeutics, FDA

- Goal and Overview of the Workshop
- FDA Requirements: They are different and why investigators need to know

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### Module I: Study Design and Beyond

**8:10-9:10 am**

### 1.1 FDA Perspective of Study Design and Plan

Speaker: Lynne Yao, M.D. (CDER)

#### Discussion topics:

- Basic elements of successful trials: why open label trials are not FDA’s standard and robust trials are needed.
- What is a well controlled trial?
- Issues of unvalidated endpoints in pediatrics
- Discussion : what is substantial evidence?
- Common errors made by clinical investigators from an FDA perspective
- Prelude to extrapolation

**9:10-10:00 am**

### 1.2 Extrapolation and Innovative Study Design

#### Pediatric Extrapolation (25 min)

Speakers: Hari Sachs, M.D. (CDER) and Dianne Murphy, M.D. (OC)

#### Innovative Study Design (25 min)

Speakers: Anne Pariser, M.D. (CDER)

**10:00-10:15 am**

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### Break

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<b>10:15-10:45 am</b>	<b>1.3 Ethics</b>  <b>What do you need to know about special protections for pediatric subjects?</b> Speaker: Robert (Skip) Nelson, M.D., Ph.D. (OC)
<b>10:45-11:25 pm</b>	<b>1.4 Get the Dosing Right</b>  <b>What Do We Look for to Support Pediatric Dosing?</b> (20 min) Speaker: Lily Mulugeta, PharmD. (CDER)  <b>Innovation, Challenges and Lessons from the Pediatric Trials Network</b> (20 min) Speaker: Brian Smith, M.D., M.P.H., M.H.S. (Duke)
<b>11:25-11:55 pm</b>	<b>1.5 Impacts of Pediatric Specific Safety Issues on Pediatric Trial Conduct</b>  <b>Pre-marketing Section</b> (15 min) <b>What Constitutes an Adequate Pediatric Safety Database?</b> Speaker: Linda Lewis, M.D. (CDER)  <b>Post-marketing Section</b> (15 min) <b>Gathering Adequate Clinical Trial Data for the Postmarketing Safety Database?</b> Speaker: Andrew Mosholder, M.D., M.P.H. (CDER)
<b>11:55-12:15 pm</b>	<b>1.6 Key Elements of Statistical Analyses for Studies with Small Populations (Pediatrics)?</b> Speaker: Lisa A. Kammerman, Ph.D. (AstraZeneca)
<b>12:15-1:00 pm</b>	<hr/> <b>Lunch</b> (on your own) <hr/>
<b>1:00-2:00 pm</b>	<b>Post – Module I Discussion</b> Chairs: Dianne Murphy, M.D. and Lynne Yao, M.D.  <hr/> <b>Module II: Formulations, CMC and Inspections (GCP)</b>
<b>2:00-2:30 pm</b>	<b>2.1 Formulations and CMC</b> <b>Pediatric Dosage Development: Where Are We?</b> Speaker: Julia Pinto, Ph.D. (CDER)
<b>2:30-3:15 pm</b>	<b>2.2 What is GCP?</b> <b>How to Survive an FDA Inspection</b> Speaker: Susan Leibenhaut, (CDER)
<b>3:15-3:30 pm</b>	<hr/> <b>Break</b> <hr/>

**3:30-4:00 pm**      **Module III: Non-clinical Study(ies)**  
Speaker: Melissa Tassinari, Ph.D. (CDER)

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**Module IV: Written Request Processes and NIH Real-life Experiences**

**4:00-4:15 pm**      **4.1 Written Requests and the NIH Process: Changing from Reactive to Proactive**  
Speaker: Dianne Murphy, M.D. (OC)

**4:15-5:00 pm**      **4.2 NIH Real-Life Experiences**  
Speaker: Anne Zajicek, M.D., PharmD. (NIH)

**Discussion topics:**

- NIH and need for reproducibility of scientific findings
- NIH/FDA product development activities directed to product labeling, publication of trial results and data availability
- High quality clinical trial designs directed at meeting FDA standards – Best Pharmaceuticals for Children Act experiences
- Need for quality data that is packaged so it can be analyzed on a patient level and can be combined with other data
- Communication with regulators: what are the options, what types of meetings and interactions, expectations, goals and best practices

**5:00-5:15 pm**      **4.3 Network Collaboration**  
**Global Network Collaborations**  
Speaker: Jonathan Davis, M.D. (Tufts)

**Discussion topics:**

- Networks and increasing integration and global collaboration between academia, industry, FDA, NIH, and community groups
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**5:15-5:30 pm**      **Wrap-up**  
Speakers: Dianne Murphy/Lynne Yao/Anne Zajicek/Jonathan Davis

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**5:30 pm**      **Adjourn**