



## **PUBLIC WORKSHOP ADVANCING THE DEVELOPMENT OF PEDIATRIC THERAPEUTICS: PEDIATRIC BONE HEALTH**

Tuesday, June 3, 2014  
Silver Spring, MD

### **AGENDA**

**8:00-8:10am**      **Welcome and Introductory Remarks**

Lynne Yao, M.D., Associate Director, Office of New Drugs (OND), Pediatric and Maternal Health Staff (PMHS), Center for Drug Evaluation and Research (CDER), FDA

**8:10-9:30am**      **Session I: Bone Signals From Animal and Adult Human Studies**

Session Chairs: Melissa Tassinari, Ph.D., Senior Clinical Advisor, PMHS, OND, CDER, FDA, and Gemma Kuijpers, Ph.D., Pharmacologist, Division of Bone, Reproductive and Urologic Products (DBRUP), OND, CDER, FDA

**Presentations**

**Signals for Bone Toxicity in Nonclinical Safety Assessments and Regulatory Applications for Pediatric Trials**

Melissa Tassinari, Ph.D.

**Nonclinical and Clinical Assessment of Bone Toxicity with Tenofovir Disoproxil Fumarate**

Linda Lewis, M.D., Medical Officer Team Leader, Division of Anti-Viral Products (DAVP), OND, CDER, FDA

**FDA Experience With Esomeprazole Strontium**

Robert Fiorentino, M.D., M.P.H, Medical Officer Team Leader, Division of Gastroenterology and Inborn Errors Products (DGIEP), OND, CDER, FDA

**Discussion Topics**

- Discuss the adequacy of standard nonclinical studies to provide information on bone effects of drugs and biologics
- Discuss signals from animal and from adult human studies that would prompt bone safety studies in children
- Discuss the impact of different levels of evidence on the decision to evaluate bone health in children (i.e., adult animal vs. juvenile animal data; animal vs. human data; uncontrolled vs. controlled adult data).

**9:30-10:00am Break**

**10:00-11:30am Session II: Biomarkers and Evaluation of Pediatric Bone Health**

Chair: Theresa Kehoe, M.D.; Medical Officer Team Leader, Division of Bone, Reproductive and Urologic Products (DBRUP), OND, CDER, FDA

**Presentations**

**Bone Mineral Density in Childhood Study: Foundation for Studies in Pediatric Bone Disease**

Karen Winer, M.D., Director, Endocrinology and Bone Programs  
Pediatric Growth and Nutrition Branch (PGNB), The Eunice Kennedy Shriver  
National Institute of Child Health and Human Development, NIH

**Use of Biomarkers in Clinical Trials**

Lynne Yao, M.D.

**Bone Biomarkers**

Stephen Voss, M.D., Medical Officer, DBRUP, OND, CDER, FDA

**Noninvasive Assessment of Bone Health in the Pediatric Population**

Mary Leonard, M.D., Director of Research, Division of Nephrology, Professor  
of Pediatrics and Epidemiology, Perelman School of Medicine at the  
University of Pennsylvania

**Discussion Topics**

- Discuss bone biomarkers (radiographic and others) that may be potentially useful in evaluating pediatric bone health
- Discuss additional clinical information that may be needed in order to rely on bone biomarkers as clinical endpoints in evaluation of pediatric bone health
- Discuss future directions for pediatric bone health biomarker research

**11:30-12:30pm Lunch (Cafeteria on campus)**

**12:30-4:30pm Session III: Tools and Strategies in the Evaluation of Pediatric Bone Health**

Chairs: Lynne Yao, M.D., Jean Temeck, M.D., Medical Officer, Supervisory,  
Office of Pediatric Therapeutics (OPT), Office of the Commissioner (OC), FDA;  
and Theresa Kehoe, M.D.

**Presentations**

**Design Considerations in Studies Evaluating Bone Health in Children**

Simone Pinheiro, ScD, MScALM, Associate Director, Division of Epidemiology-  
1, Office of Pharmacovigilance and Epidemiology (OPE), CDER, FDA

**Evaluation of the Systemic Effects of Locally Acting Corticosteroids**

Miya Paterniti, M.D., Medical Officer, Division of Pulmonary, Allergy and Rheumatology Products (DPARP), OND, CDER, FDA

**Steroids and Bone Health in Pediatric Patients: Dose Response Relationships and Clinical Relevance**

Gilbert Burckart, Pharm.D., Associate Director for Pediatrics, OCP, OTS, CDER, FDA

**Use of Proton Pump Inhibitors and Risk of Bone Fractures in Adults**

Tamara Johnson, M.D., M.S., Medical Officer, PMHS, OND, CDER, FDA

**Monitoring Bone Toxicity in Pediatric Clinical Trials of Tenofovir Disoproxil Fumarate**

Linda Lewis, M.D.

**2:10-2:30pm**      **Break**

**Discussion Points**

- Discuss potential overall study design(s) to assess short-term and long-term bone health in children
- Discuss potential endpoints that may be used in short-term and longer-term studies to evaluate pediatric bone health
- Discuss the current level of evidence to support use of specific radiographic biomarkers in short and longer-term studies of pediatric bone health
- Discuss the current level of evidence to support use of specific biochemical biomarkers in short and longer-term studies of pediatric bone health
- Discuss the appropriate length of studies to assess short-term and long-term effects on pediatric bone health
- Discuss optimal periods during growth and development to evaluate pediatric bone health

**4:30-5:00pm**      **Conclusions, Summary of Morning and Afternoon sessions and Next Steps**  
Jean Temeck, M.D.

**5:00pm**      **Adjourn**