

# Third Meeting of the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children

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**Thursday, January 16, 2014**

10:30 AM – 11:00 AM

## **Welcome**

- Opening Remarks
- Committee Correspondence
- Approval of September 2013 Meeting Minutes  
*\*Vote\**

Joseph Bocchini, MD  
Committee Chair

Debi Sarkar, MPH  
Designated Federal Official

11:00 AM – 11:20 AM

## **Update from the Sickle Cell Disease and Screening for Trait in Athletes Ad-hoc Workgroup**

Alexis Thompson, MD  
Committee Member

11:20 AM – 11:30 AM

## **Introduction to Challenges When Conducting Pilot Studies and Possible Solutions**

Jeff Botkin, MD  
Committee Member

11:30 AM – 12:30 PM

## **Update on the Newborn Screening TA Center – NewSTEPS**

Marci Sontag, PhD  
Associate Director of NewSTEPS  
Colorado School of Public Health

Jelili Ojodu, MPH  
Director, Newborn Screening and Genetics  
Association of Public Health Laboratories

12:30 PM – 1:30 PM

## **LUNCH**

1:30 PM – 1:45 PM

## **PUBLIC COMMENTS**

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## **Thursday, January 16, 2014 (cont'd)**

1:45 PM – 2:30 PM

### **X-linked Adrenoleukodystrophy (ALD) – Update from Nomination and Prioritization Workgroup**

Dietrich Matern, MD  
Committee Member

*\* Vote on whether or not to move ALD forward to the Condition Review Team\**

2:30 PM – 4:30 PM

### **Subcommittee Meetings**

- *Education and Training*
- *Follow-up and Treatment*
- *Laboratory Standards and Procedures*

## **Friday, January 17, 2014**

10:00 AM – 11:00 AM

### **Newborn Screening Specimen Transport – Update**

Susan Tanksley, PhD  
Co-Chair, Laboratory Standards and Procedures Subcommittee  
Association of Public Health Laboratories  
Organizational Representative

11:00 AM – 12:00 PM

### **Mucopolysaccharidosis type 1 (MPS-1) – Update from Condition Review Team**

Alex Kemper, MD, MPH, MS  
Condition Review Workgroup  
Duke Clinical Research Institute and Department of Pediatrics

12:00 PM – 1:00 PM

**LUNCH**

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**Friday, January 17, 2014 (cont'd)**

1:00 PM – 2:00 PM

## **Subcommittee Reports**

- *Education and Training*  
Don Bailey, PhD, MEd  
Chair  
Committee Member  
  
Beth Tarini, MD, MS, FAAP  
Co-Chair  
American Academy of Pediatrics  
Organizational Representative
- *Follow-up and Treatment*  
Carol Greene, MD  
Chair  
Society for Inherited Metabolic Disorders  
Organizational Representative  
  
Chris Kus, MD, MPH  
Co-Chair  
Association of State & Territorial Health  
Officials Organizational Representative
- *Laboratory Standards and Procedures*  
Kellie Kelm, PhD  
Chair  
Committee Member  
  
Susan Tanksley, PhD  
Co-Chair  
Association of Public Health Laboratories  
Organizational Representative

2:00 PM – 2:10 PM

## **Discussion on Future Meeting Topics**

2:10 PM

**ADJOURN**