Thursday, February 12, 2015 – Day 1

8:30 AM – 9:00 AM  
**Welcome**
- Opening Remarks
- Committee Correspondence
- Establishment of statutory advisory committee
- Approval of September 2014 Meeting Minutes
  *
  *Vote*

Joseph Bocchini, MD  
Committee Chair  
Louisiana State University

Debi Sarkar, MPH  
Designated Federal Official  
Health Resources and Services Administration

9:00 AM – 9:30 AM  
**U.S. Preventive Services Task Force – Overview and the transfer of newborn screening topics to the DACHDNC**

Iris R. Mabry-Hernandez, MD, MPH  
Medical Officer  
U.S. Preventive Services Task Force Program  
Center for Evidence and Practice Improvement  
Agency for Healthcare Research and Quality

9:30 AM – 10:30 AM  
**Pilot Study Workgroup Update**

*Panel of Speakers include –*
Jeff Botkin, MD  
Committee Member and Chair of the Pilot Study Workgroup
Carla Cuthbert, PhD, FACMG, FCCMG  
Chief, Newborn Screening and Molecular Biology  
Branch, Division of Laboratory Sciences, National  
Center for Environmental Health  
Centers for Disease Control and Prevention  

Tiina Urv, PhD  
Program Director  
Intellectual and Developmental Disabilities Branch  
Eunice Kennedy Shriver National Institute of Child  
Health and Human Development  
National Institutes of Health  

Michael Watson, PhD, FACMG  
Organizational Representative  
American College of Medical Genetics  

Anne Comeau, PhD  
Deputy Director, New England Newborn Screening  
Program  
Professor, Department of Pediatrics,  
UMass Medical School  

10:30 AM – 10:45 AM  
Public Comments  

10:45 AM – 11:00 AM  
Break  

11:00 AM – 11:30 AM  
Laboratory Procedures and Standards  
Subcommittee Update – Timely Newborn  
Screening Project and other projects  
*Vote on Final Recommendations*  

Kellie Kelm, PhD  
Chair  
Ex-Officio Committee Member  
Food and Drug Administration  

Susan Tanksley, PhD  
Co-Chair  
Organizational Representative  
Association of Public Health Laboratories
11:30 AM – 11:40 AM  
**Condition Review Process – Overview**  
Joseph Bocchini, MD  
Committee Chair  
Louisiana State University

11:40 AM – 11:50 AM  
**Evaluating Harms in the Assessment of Net Benefits: A Framework for Newborn Screening Condition Review**  
Nancy Green, MD  
Condition Review Workgroup  
Columbia University Medical Center

11:45 AM – 12:15 PM  
**Condition Review Update – Adrenoleukodystrophy (ALD)**  
Alex Kemper, MD, MPH, MS  
Condition Review Workgroup  
Duke Clinical Research Institute and Department of Pediatrics

12:15 PM – 12:45 PM  
**LUNCH**

12:45 PM – 2:45 PM  
**WORKING LUNCH**

12:45 PM – 2:45 PM  
**Cost and Cost-Effectiveness Analysis**  
Scott Grosse, PhD  
Senior Health Economist  
National Center on Birth Defects and Developmental Disabilities  
Centers for Disease Control and Prevention  
Alex Kemper, MD, MPH, MS  
Condition Review Workgroup  
Duke Clinical Research Institute and Department of Pediatrics  
Lisa Prosser, PhD, MS  
Condition Review Workgroup  
Child Health Evaluation and Research Unit  
University of Michigan
Discretionary Advisory Committee on Heritable Disorders in Newborns and Children February 2015 Meeting

2:45 PM – 3:15 PM  BREAK

3:15 PM – 5:00 PM  SUBCOMMITTEE MEETINGS

Friday, February 13, 2015 – Day 2

9:00 AM – 9:05 AM  WELCOME

Joseph Bocchini, MD
Committee Chair

9:05 AM – 9:20 AM  PUBLIC COMMENTS

9:20 AM – 10:30 AM  Final Condition Review of Mucopolysaccharidosis I (MPS I)

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

10:30 AM – 10:45 AM  BREAK

10:45 AM – 11:45 AM  Final Condition Review of Mucopolysaccharidosis I - continued

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

11:45 AM – 1:00 PM  LUNCH
### Committee Review of Mucopolysaccharidosis I

1:00 PM – 2:00 PM

Jeff Botkin, MD, MPH  
Committee Member  
University of Utah

Stephen McDonough, MD  
Committee Member  
Sanford Health Bismarck

*Vote – To recommend or not recommend to the Secretary the addition of Mucopolysaccharidosis I to the Recommended Uniform Screening Panel*

### Follow-up and Treatment Subcommittee Update

2:00 PM – 2:15 PM

Charlie Homer, MD, MPH  
Chair  
Committee Member  
National Institute for Children’s Health Quality

### Education and Training Subcommittee Update

2:15 PM – 2:30 PM

Cathy Wicklund, MS, CGC  
Chair  
Committee Member  
Northwestern University

### Laboratory Procedures and Standards Subcommittee Update

2:30 PM – 2:45 PM

Kellie Kelm, PhD  
Chair  
Ex-Officio Committee Member  
Food and Drug Administration

### Future Topics – Discussion

2:45 PM – 3:00 PM

### Adjourn

3:00 PM