

Discretionary Advisory Committee on Heritable Disorders in Newborns and Children February 2015 Meeting

February 12 – 13, 2015
Webinar and In-Person Meeting
5635 Fishers Lane
Rockville, Maryland 20857

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

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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

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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

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

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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

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1:00 PM – 2:00 PM

Committee Review of Mucopolysaccharidosis I

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***

2:00 PM – 2:15 PM

Follow-up and Treatment Subcommittee Update

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

2:15 PM – 2:30 PM

Education and Training Subcommittee Update

Cathy Wicklund, MS, CGC
Chair
Committee Member
Northwestern University

2:30 PM – 2:45 PM

Laboratory Procedures and Standards Subcommittee Update

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

2:45 PM – 3:00 PM

FUTURE TOPICS – DISCUSSION

3:00 PM

ADJOURN