

# 24th Meeting of the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children

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Renaissance Washington, DC, Dupont Circle Hotel  
May 5-6, 2011

## Thursday, May 5, 2011

- 8:30 A.M. – 9:30 A.M.**      **Continental Breakfast (Committee Members and Presenters)**
- 9:30 A.M. – 10:00 A.M.**      **Committee Business**
- Approval of Minutes from the January 2011 Meeting
  - Introduction of New Members
  - Committee Correspondence
- R. Rodney Howell, M.D.**  
Committee Chair
- 10:00 A.M. – 11:30 A.M.**      **Public Health Framework for Hospital-based Newborn Screening and Committee Discussion**
- Coleen Boyle, Ph.D., M.S. and Jeffrey Botkin, M.D., M.P.H.**  
Committee Members
- Major Issues**
- Nancy S. Green, M.D.**  
Columbia University Medical Center
- Perspectives from Committee Members and Organization Representatives**
- Committee Discussion**
- 11:30 A.M. – 12:00 P.M.**      **Public Comments**
- 12:00 P.M. – 1:00 P.M.**      **Lunch**

**1:00 P.M.—2:00 P.M.**

**Food and Drug Administration Policies and Procedures Relevant to  
Individuals with Rare Heritable Disorders**

**Gerard Vockley, M.D., Ph.D.**  
Committee Member

**Expanded Access to Investigational Drugs: the Road to Marketing**

**Tim Cote, MD MPH**  
Director, FDA Office of Orphan Products Development

**Anne Pariser, M.D.**  
Associate Director for Rare Diseases, FDA Center for Drug  
Evaluation and Research

**Critical Drug Shortages for Necessary Medications**

**CAPT Valerie Jensen R. Ph.**  
Associate Director Drug Shortage Program, FDA Center for Drug  
Evaluation and Research

**Overview of Device Regulation**

**Kellie Kelm, Ph.D.**  
Committee Member

**2:00 P.M. – 5:00 PM**

**Subcommittee Meetings**

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

**Friday, May 6, 2011**

- 7:30 A.M. – 8:30 A.M. Continental Breakfast (Committee Members and Presenters)**
- 8:30 A.M. –10:00 A.M. Subcommittee Reports**
- **Subcommittee on Laboratory Standards and Procedures**  
**Gerard Vockley, M.D., Ph.D.**  
Committee Member
  - **Subcommittee on Education and Training**  
**Don Bailey, Ph.D., M.Ed. and Tracy L. Trotter, M.D.**  
Committee Members
  - **Subcommittee on Follow-up and Treatment**  
**Coleen Boyle, Ph.D., M.S. and Jeffrey Botkin, M.D., M.P.H.**  
Committee Members
  - **Report from the Evidence Evaluation Methods Workgroup Meeting**  
**Ned Calonge, M.D., M.P.H.**  
Committee Member
- 10:00 A.M. – 10:30 A.M. Break**
- 10:30 A.M –12:00 P.M. Evidence Review Workgroup Report: Final Report on the Candidate Nomination Hyperbilirubinemia and Committee Discussion**
- John Co, M.D., M.P.H.**  
SACHDNC External Evidence Review Workgroup
- Gerard Vockley, M.D., Ph.D.**  
Committee Member
- 12:00 – 1:00 P.M. Lunch**
- 1:00 P.M. – 1:45 P.M. SCID Report to the Secretary and Committee Discussion**
- Amy M. Brower, PhD**  
Project Manager, NCC-LTFU|NBSTRN
- 1:45 P.M. – 2:30 P.M. The Committee’s Annual Report to Congress and Committee Discussion**
- Alissa Johnson, M.A.**  
Johnson Policy Consulting
- Alaina Harris, M.S.W., M.P.H.**  
Committee Staff
- 2:30 P.M. – 3:00 P.M. Committee Discussion**
- R. Rodney Howell, M.D.**  
Committee Chair
- 3:00 P.M. Adjourn**