

Committee Related Work



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May 9, 2016

Welcome

- **May 2016 Meeting of the Advisory Committee on Heritable Disorders in Newborns and Children**
- **New AMCHP Representative - Kate Tullis, PhD**
- **Roll Call**

ACHDNC Business

■ Secretarial Correspondence

- MPS I and X-ALD
Recommendations

- Accepted

- Funding Recommendations

- Not accepted

■ RUSP – 34 conditions

ACHDNC Business

■ Funding Recommendations

- Secretary encouraged federal agencies to continue to provide technical assistance and support states with existing resources
- As a result:
 - HRSA Funding Opportunity Announcement
 - HRSA-16-062: Newborn Screening Implementation Program Regarding Conditions Added to the Recommended Uniform Screening Panel (RUSP)

2017 Nominations for Committee Membership

- **Nominations are currently due on May 16.**
- **Nominations are being sought to fill three Committee positions for individuals who are:**
 - **Medical, technical, public health, or scientific professionals with special expertise in the field of heritable disorders or in providing screening, counseling, testing, or specialty services for newborns and children at risk for heritable disorders;**
 - **Experts in ethics and infectious diseases and who have worked and published material in the area of newborn screening;**
 - **Members of the public having special expertise about or concern with heritable disorders; or**
 - **Members from such federal agencies, public health constituencies, and medical professional societies as determined to be necessary by the Secretary.**
- **Submit all required information electronically as email attachments to Ms. Alaina Harris, Genetic Services Branch, Maternal and Child Health Bureau, HRSA (aharris@hrsa.gov).**

Vote

**ACHDNC FEBRUARY 2016
MEETING MINUTES**

ACHDNC Priorities

- **Pilot Study Workgroup – report and recommendations**
- **Cost Analysis Workgroup – update**
- **Timeliness Workgroup – update**

ACHDNC Standing Workgroups (Subcommittees)

- **Name change**
 - **Education and Training Workgroup**
 - **Follow up and Treatment Workgroup**
 - **Laboratory Standards and Procedures Workgroup**
- **Aligns with Federal Advisory Committee Act requirements**
- **Workgroups will meet this afternoon**

Workgroup Projects

Education and Training

- Create companion piece to ACT sheets that provides PCPs with guidance and tips for discussing positive NBS results with parents
- Educational Outreach Project in collaboration with NBS Clearinghouse/Baby's First Test

Follow up and Treatment

- Promoting the role of clinical quality measures to promote LTFU
- Examine State Infrastructure for LTFU*

Workgroup Projects

Laboratory Standards and Procedures

- Define and implement a mechanism for the periodic review and assessment of laboratory procedures utilized for effective and efficient testing of the conditions included in the uniform panel.
- Define and implement a mechanism for the periodic review and assessment of infrastructure and services needed for effective and efficient screening of the conditions included in the uniform panel.

CY 2016 Meetings

- **August 25 and 26, 2016**
(Location: Rockville, MD) and
Via Webcast
- **November 3 and 4, 2016**
(webinar)

May 2016 Meeting Topics

- **Continue discussion on long-term follow up in NBS**
 - Medical foods impact on patient access
- **Guanidinoacetate methyltransferase deficiency (GAMT)**
 - Update from N&P Workgroup and vote
- **Prenatal Education about NBS and Dried Bloodspots**
- **Workgroup Updates**

Ethics and COIs

- **When to recuse:** In all matters likely to affect the financial interests of any organization with which you serve as an officer, director, trustee, or general partner unless you are also an employee of the organization or unless you have received a waiver from HHS authorizing them to participate.

Meeting Participation

- All federal committees governed by the Federal Advisory Committee Act (FACA), Public Law 92-463, as amended (5 U.S.C. App. 2), and by 41 C.F.R. Parts 101-6 and 102-3
- FACA sets the standards for the establishment, utilization, and management of Federal Advisory committees

Meeting Participation

- All Committee meetings are open to the public.
- If the public wish to participate in the discussion, the procedures for doing so are published in the Federal Register and/or announced at the opening of a meeting.
- With advance approval of the Chair or DFO, public participants may question committee members or other participants
- May submit written statements.
- Also, public participants should be advised that committee members are given copies of all written statements submitted to the public;
- Any further public participation will be solely at the discretion of the Chair and DFO

Webcast

- Remember to state your name first to ensure proper recording for the Committee transcript and minutes.

Kathryn Camp, MS, RD, CSP(C)
Office of Dietary Supplements
National Institutes of Health

MEDICAL FOODS FOR INBORN ERRORS OF METABOLISM: ISSUES IN PATIENT ACCESS