

Committee Related Work: Preparing for the Transition



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September 23, 2011

Secretary's Advisory Committee on Heritable Disorders in Newborns and Children

- Chartered in 2003 – **Section 1111** of the Public Health Services Act
- Charter updated in the Newborn Screening Saves Lives Act of 2008 (in reauthorization of PHS Act)
 - Extended operation during a 5 year period beginning April 24, 2008
 - **Reauthorization required in 2013**

SACHDNC Charter

■ Objective and Scope of Activities

The Committee provides advice to the Secretary about aspects of newborn and childhood screening and technical information for the development of policies and priorities that will enhance the ability of the State and local health agencies to provide for newborn and child screening, counseling and health care services for newborns and children having or at risk for heritable disorders

SACHDNC Charter

■ Duties

- Establish by-laws to specify the Committee's operating procedures
- Review and report regularly on newborn and childhood screening practices
- Recommend improvements in the national newborn and childhood screening programs

Activities impacting and complimenting the Committee work

- **Section 1112** – establishes a clearinghouse of newborn screening
- **Section 1113** – establishes a program for laboratory quality
- **Section 1114** – establishes an Interagency Coordinating Committee on Newborn and Child Screening
- **Section 1116** – establishes the Hunter Kelly newborn screening research program in NICHD

SACHDNC Charter

- **Section 1109** of the Children's Health Act of 2000 establishes grant programs to improve the ability of States to provide newborn and child screening for heritable disorders
- **SACHDNC provides:**
 - Advice and recommendations to the Secretary concerning grants and projects awarded or funded under **section 1109**
 - Technical information to the Secretary for the development of policies and priorities for the administration of grants under **section 1109**

SACHDNC Charter: duties

- **Makes systematic evidence-based and peer-reviewed recommendations that include the heritable disorders that have the potential to significantly impact public health for which all newborns should be screened, including secondary conditions that may be identified as a result of the laboratory methods used for screening**

SACHDNC Charter: duties

- Develop a model decision-matrix for newborn screening expansion, including an evaluation of the potential public health impact of such expansion, and periodically evaluate and update the recommended uniform screening panel, as appropriate, based on such decision-matrix

SACHDNC Charter: duties

- Consider ways to ensure that all States attain the capacity to screen for the conditions chosen
 - include in such considerations, the results of grant funding under **section 1109**
- Provide such recommendations, advice or information as may be necessary to enhance, expand or improve the ability of the Secretary to reduce the mortality or morbidity from heritable disorders, which may include.....

SACHDNC Charter: duties

- ...recommendations, advice or information dealing with:
 - **Follow-up activities**, including making a rapid diagnosis in short-term and those that ascertain long-term case management outcomes and appropriate access to services
 - **Implementation, monitoring and evaluation of newborn screening activities** including diagnosis, screening, follow-up, and treatment activities
 - **Diagnostic and other technology** used in screening

SACHDNC Charter: duties

- **The availability and reporting of testing for conditions for which there is no existing treatment**
- **Conditions not included in the recommended uniform screening panel that are treatable with FDA-approved products or other safe treatments, as determined by scientific evidence and peer review**
- **Minimum standards and related policies and procedures used by State newborn screening programs such as**
 - **Language, terminology, standardized case definitions**

SACHDNC Charter: duties

- **Quality assurance, oversight and evaluation of State newborn screening programs, including ensuring that tests and technologies used by each State meet established standards for detecting and reporting positive screening results**
- **Public and provider awareness and education**
- **Cost and effectiveness of newborn screening and medical evaluation systems and intervention programs conducted by State-based programs**

SACHDNC Charter: duties

- Identification of the causes of, public health impacts of, and risk factors for heritable disorders
- **Coordination of surveillance activities**, including standardized data collection and reporting, harmonization of laboratory definitions for heritable disorders and testing results, and confirmatory testing and verification of positive results

SACHDNC Charter: duties

■ Reporting requirements

- Publish an annual report on peer-reviewed newborn screening guidelines, including follow-up and treatment in the US**
- Submit such report to the appropriate committees of Congress, the Secretary, the Interagency Coordinating Committee and the State departments of health**
- Disseminate such report, including through posting on the internet clearinghouse**

SACHDNC

■ Current Subcommittees

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

■ Current working groups

- Nominations and Prioritization
- Evidence Review (external)
- Specific topic related: Carrier Screening, Newborn Screening Education, Evidence Evaluation Methods

SACHDNC: needs to be addressed/considered

- **Assign members to the Nominations and Prioritization working group**
- **Review structure and function of each of current standing working groups**
- **Prepare for reauthorization in 2013**
 - **Review of legislation and charter**
 - **Determine if SOPs and Committee activities match all duties outlined in charter**

SACHDNC: needs to be addressed/considered

- **Develop a matrix for evaluation of public health impact**
 - **Benefits**
 - **Cost-effectiveness**
 - **Modeling, use of health economists**
 - **Technical aspects**
 - **Laboratory capacity**
 - **Provider capacity**

“In addition, I am requesting that the SACHDNC collaborate with HRSA to complete a thorough evaluation of the potential public health impact of universal screening for CCHD, as required by the authorizing statute, section 1111 of the PHS Act (42 U.S.C. § 300b-10(b)(4)).”¹

¹ From the Secretary’s letter to Dr. Howell, Sept. 21,2011 approving SACHDNC’s CCHD recommendations

“Specifically, it would be beneficial to states, health care facilities, and individual clinicians to have the SACHDNC and other public health experts, partner with HRSA to provide information about a number of issues, including but not limited to the following:

- What will be the impact on state health departments, including staffing needs, to implement this program? what are the roles of the state health departments?**
- What capability is present to ensure that all babies are screened and their results are communicated to providers, including assuring that those not screened at birth receive a screen?”¹**

¹ From the Secretary’s letter to Dr. Howell, Sept. 21,2011 approving SACHDNC’s CCHD recommendations

SACHDNC: needs to be addressed/considered

- **Follow-up on policy decisions**
 - **Implementation**
 - **Surveillance**
 - **Patient outcome data**
 - **Diagnosis**
 - **Short-term and long-term case management outcomes**
 - **Appropriate access to services**
 - **Evaluation**
- **Planned policy reviews**

SACHDNC: needs to be addressed/considered

- **Review structure and function of working groups for individual nominated disorders accepted for review**
 - **Make up**
 - **SOP**
 - **Interactions with evidence review team**
 - **Work product development**
 - **Format of final product**
 - **recommendations**
 - **Presentation to full Committee for discussion and vote**

SACHDNC: needs to be addressed/considered

- **Project/product developed by groups outside of the Committee that enhance or support the work of the Committee**
 - **What should be the process of review of those products?**
 - **How should the products submitted to the Committee, if deemed appropriate, be addressed**
 - **Endorsement vs Supported vs Approval**

Discussion