

**Secretary's Advisory Committee on
Heritable Disorders in
Newborns and Children (SACHDNC)**

**Activities Dealing with Medical Foods
For Children with Metabolic Conditions**

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SACHDNC

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



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

- **Legislative Authority:** Established under Title XXVI of the Children's Health Care Act of 2000 "Screening for Heritable Disorders"; amended by the **Newborn Screening Saves Lives Act of 2008**.
- The Committee advises and guides the Secretary regarding the most appropriate application of universal newborn screening tests, technologies, policies, guidelines and programs for effectively reducing morbidity and mortality in newborns and children having or at risk for heritable disorders.
- The Act reauthorizes and expands the role of the Committee to include: (1) making recommendations that include heritable disorders for which all newborns should be screened; (2) developing a model decision-matrix for newborn screening; and (3) considering ways to ensure that all States attain the capacity to screen for the recommended conditions.
- **Web site:** <http://www.hrsa.gov/heritabledisorders.committee/>

SACHDNC Activities Related to Medical Foods

- **Follow-up and Treatment Subcommittee created the Medical Foods Expert Panel in 2007.**
 - **To acknowledge strong support and advocacy in providing a SACHDNC forum to address issues of coverage and access to Medical Foods.**
 - **To develop recommendations to the Committee to overcoming identified barriers in order to improve short- and long –term follow-up of newborn screening results; a subcommittee charge.**

Challenges: Medical Foods Coverage

- **Treatment includes: Medical foods, modified low protein foods, nutritional supplements, and feeding supplies.**
- **State mandates and programs vary widely: Private insurance and assistance through public programs (Medicaid, WIC).**
- **Reimbursement to parents: Barriers and challenges to receiving reimbursement for products ostensibly covered by insurance companies.**
- **Inadequate coverage of essential treatment: Jeopardizes health and well being of children with inborn errors of metabolism.**

Challenge: FDA Medical Food Definition

- **“a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.”**
 - **As defined in section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3)). This legal definition of a medical food was added to the Orphan Drug Amendments of 1988**

SACHDNC Medical Foods Activities

- **Review of Position Statements** calling for appropriate reimbursement of medical foods - AAP, SIMD and GMDI.
- **Medical Foods Workgroup meeting: Insurance Coverage of Medical Formulas, Low Protein Foods, Nutritional Supplements and Related Supplies, June 2, 2008.**
 - Private and public insurance perspectives, and Employment-Based Health Plan Issues.
- **State Statutes and Regulations on Dietary Treatment of Disorders Identified Through Newborn Screening, ACMG, July 2008, HRSA funded. Presented to Committee.**
- **Medically Necessary Foods Act of 2009**, drafted for the treatment of conditions recommended by the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC). Currently, A BILL to provide for the coverage of medically necessary food under Federal health programs and private health insurance. Senator Kerry will re-introduce Bill in February 2011. KER09804
- **Affordable Care Act (ACA).** Presented to the Committee, January 18, 2011

Committee and HHS Secretary Correspondence

- **Committee Recommendation to the HHS Secretary, May 19, 2009**
- **Initiate appropriate action to address the gaps in coverage and lack of uniformity in reimbursement of medical foods and foods that are critical treatments for children and adults identified with inborn errors of metabolism through newborn screening.**
 - **1) A more uniform approach toward coverage by health care payers of medical foods and foods for those conditions recommended by the Committee, including Federal insurance programs - CHIP, Tricare, and Medicaid; and plans under the Employment Retirement Income Security Act (ERISA).**
 - **2) Specific amendments to Medicaid legislation to ensure more uniform coverage by State Medicaid programs.**
 - **3) A minimum yearly coverage with age-specific minimum levels set for all health insurance plans, including CHIP, Tricare, Medicaid.**

Committee and HHS Secretary Correspondence

- **The HHS Secretary response to the Committee, Dec 14, 2010.**
 - **The HHS Secretary is unable to adopt the Committee recommendations at this time.**
 - **Department of Labor (DOL) is conducting a survey to inform our determination of a typical employer-sponsored plan. Survey results are expected in March 2011.**
 - **Institute of Medicine (IOM) will hold a public workshop in early 2011 on essential health benefits both initially and over time. IOM report is due at end of Sept.**
 - **The HHS Secretary is unable to make determinations about particular benefits until DOL results and IOM recommendations are received.**
 - **The HHS Secretary will give serious consideration to issues raised by SACHDNC.**

Parent Survey of Insurance Coverage of Medical Foods for Children with Metabolic Conditions, 2008-2010

- **Purpose:** to survey parents of children (from birth through 17 years of age) with metabolic disorders to obtain information on the status of current insurance coverage and access to coverage.
- **Information sought:**
 - 1) Needs of children for metabolic foods and formulas, modified low protein foods, prescribed dietary supplements, and medical feeding supplies and equipment.
 - 2) Proportion of expenses paid by insurance coverage for those medical foods items.
 - 3) Out-of-pocket expenses for foods and formulas.
 - 4) Health plan caps on the coverage provided.
- **Rationale:** to inform federal and state public health policy decisions aimed at reducing financial barriers and improving coverage of medical foods for the treatment of children with inborn errors of metabolism.

Medical Foods Survey Implementation

- **HRSA MCHB GSB Regional Genetics and Newborn Screening Service Collaboratives: Heritable Disorders Program**
 - **Region 2 New York Mid-Atlantic Consortium for Genetic and Newborn Screening**
 - **Region 3 Southeast NBS and Genetics Collaborative**
 - **Region 4 Midwest Genetics Collaborative**
- **Survey sites: genetic centers located in the 3 designated regions with IRB approval**
- **Data collection and analyses by each region; and regional data integration and analysis by HRSA MCHB.**

Summary of Survey Responses

- **305 families across 3 regions responded. One-half of children under age 5.**
- **Nearly all children had some type of health care coverage (even if it did not pay for these products).**
- **Children need lots of products for nutritional support of their conditions: medical Foods; Dietary Supplements; Modified Low Protein Foods (MLPF); and Feeding Supplements.**
- **Most children used more than one product: 80% use at least 2 of the surveyed products.**
- **Coverage was variable but there were at least some out-of-pocket expenses**
 - **For about 20% of families using Medical Foods.**
 - **For about 30% of families using supplements.**
 - **For about 35% of families using feeding supplies.**
 - **For about 60% of families using MLPF.**

Summary of Survey Responses

- Families often did not know if they had “caps” on insurance and the dollar amount of the cap.
- Families often did not know their out-of-pocket costs; this data is incomplete.
- Need-based supports are currently significant resources.
 - Depending on the State, WIC is an important source of support for families.
 - Medicaid is a critical source of support for many families.
- MLPF are particularly poorly supported.
- Though patterns of coverage varied from Region to Region, all Regions observed significant challenges to families in paying for these essential products.

Survey Acknowledgment: Genetic Centers

Region 4 Midwest

University of Minnesota
Department of Pediatrics and Genetics, Cell
Biology and Development
Division of Genetics and Metabolism

Cincinnati Children's Hospital Medical Center
Human Genetics Metabolic Diseases Clinic

Emory University
Department of Human Genetics
Division of Medical Genetics

Region 3 Southeast

Greenwood Genetics Center
Metabolic Clinic

University of Florida
Department of Pediatrics
Division of Genetics and Metabolism

University of North Carolina - Chapel Hill
School of Medicine
Division of Genetics and Metabolism

University of Tennessee College of Medicine
Bolin Center for Developmental Disabilities

Vanderbilt University Medical Center
Nutrition Clinic

Region 2 New York & Mid-Atlantic

Mount Sinai Medical Center
Department of Genetics and Genomic Sciences

Children's Hospital at Albany Medical Center
Division of Genetics

Children's Hospital of Pittsburgh
Division of Medical Genetics

Golisano Children's Hospital at Strong
Division of Pediatric Genetics

Maria Fareri Children's Hospital at Westchester
Medical Center
Pediatric Medical Genetics

University of Maryland Hospital for Children
Division of Human Genetics

**Special Acknowledgement: National Newborn Screening and Genetics
Resource Center (NNSGRC), HRSA U32MC00148.**

SACHDNC – Will Continue Activities Related to Medical Foods

- **Await further communication from the HHS Secretary pending DOL and IOM reports.**
- **Monitor progress of Medical Foods Equity Act.**
- **Monitor Benefits Package for Affordable Health Care Act.**
- **Work with FDA (Orphan Drug Products, Center for Food and Safety and Nutrition). Medical Foods may need to be an essential benefit.**
- **Publish - Access to Medical Foods and Formula Survey; manuscript in progress. Presented to the Committee, September 17, 2010.**