



## DEPARTMENT OF HEALTH AND HUMAN SERVICES

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Secretary's Advisory Committee on Heritable  
Disorders in Newborns and Children  
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April 7, 2009

The Honorable Charles E. Johnson  
Acting Secretary of Health and Human Services  
200 Independence Avenue, S.W.  
Washington, DC 20201

Dear Secretary Johnson:

The Advisory Committee on Heritable Disorders in Newborns and Children (the Committee) has been examining the issue of insurance coverage of medical foods and foods modified to be low protein for the treatment of a number of childhood disorders identified through newborn screening. While the Committee feels that comprehensive health care reform is needed to cover access to health care for the important childhood disorders identified through newborn screening, even health care reform does not address gaps in coverage for items that are a vital component of medical management but which are typically not included as “medical” services. After a comprehensive investigation by a workgroup of experts, the Committee is recommending a number of legislative and policy measures to ensure families receive insurance coverage for these essential components of treatment.

Medical foods and foods modified to be low protein are critical treatments for inborn errors of metabolism, major targets for newborn bloodspot screening. The Food and Drug Administration (FDA) defines medical foods as:

“... 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.”(section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3)))

Each year in the U.S., nearly 12,000 children are identified by newborn screening with one of the 29 disorders included in the Committee’s recommended core panel. While newborn screening yields cost savings for society as a whole, costs for the long-term treatment of these disorders can be prohibitive for families with a child diagnosed with one of these

conditions. Of particular concern is the cost of the medical foods and foods modified to be low protein essential for the treatment of many of these disorders. Medical foods and foods modified to be low protein are designed exclusively to avoid the nutrient or nutrients that a child is incapable of processing. Medical foods are expensive. For example, specialized infant formula designed for treatment of rare newborn errors of metabolism can exceed \$5,000 dollars a year; in contrast standard infant formula would cost approximately \$1,200 per year. Often, these foods are a self-pay expense for many families creating a financial burden for them. The financial challenges are even more complex as the child grows older and requires a variety of specially formulated foods.

To fill the gap for families, states use a patchwork of federal and state programs, including the Women, Infants and Children (WIC) program, the HRSA Title V Block grant funds, and funds from state's newborn screening programs. Most of these programs are needs-based, making it particularly challenging for families that do not qualify for support. Families who have private insurance repeatedly face the challenge of an insurance provider who does not recognize the need for these specialized treatments or they have a plan that requires large co-payments. A recent review of state legislation revealed that more than 30 states have some type of policy addressing insurance coverage for medical foods and foods modified to be low protein. Despite these state mandates, there continues to be widely variable reimbursement and support for the costs of these essential treatments. Part of the issue is that the provisions for which disorders are covered and what types of medical foods are allowed, differ from state to state and are variably enforced. A second critical factor is that nearly 60 percent of individuals with private insurance are covered by employment-based health plans that are exempt from state insurance laws. Similarly, Federal insurance programs, such as Tricare, are also exempt from state insurance laws.

Reimbursement for adults is even more problematic. Many state and federal programs are available only for children and end when an individual reaches the age of 18 or 21 years though their condition is life-long and the need for treatment does not change.

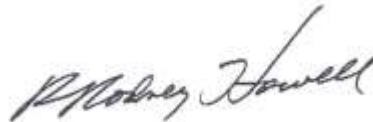
After considering the recommendations from its expert workgroup, the Committee, at its February 26, 2009 meeting, strongly and unanimously recommended that the Secretary initiate appropriate action to address medical food reimbursement for people covered by Medicaid and private insurance, including the large percentage of people in self-insured health plans offered by large companies that are exempt from state mandates. The American Academy of Pediatrics, the Society for Inherited Metabolic Disorders, and Genetic Metabolic Dietitians International all have position statements calling for appropriate reimbursement of medical foods.

Based on the review by the Committee and the recommendations from other relevant professional organizations, the Committee recommends that:

1. Federal legislation be enacted to establish a uniform requirement that health plans offer coverage of medical foods and foods modified to be low protein for those conditions recommended by the Committee. Health plans would include Federal insurance programs coverage plans (Children's Health Insurance Program, Tricare, and Medicaid) and those plans governed by the Employment Retirement Income Security Act (ERISA) and would not be subject to state exclusions.

2. Medicaid’s enabling legislation (Title XIX of the Social Security Act) be amended to ensure more uniform coverage by state Medicaid programs of medical foods and foods modified to be low protein for those conditions recommended by the Committee. (Medical foods are not mentioned in the federal Medicaid statute allowing significant variation across states with respect to the coverage of medical foods. Amending §1905(a) of the federal statute would encourage best practices and ensure greater uniformity.)
3. The following specific requirements be included in the legislation:
  - a. Medical foods (as defined by the FDA and for those conditions recommended by the Committee) delivered either orally or by tube (both are enteral) and foods modified to be low protein used under the direction of a physician for the treatment of an inborn error of metabolism should be included as medical benefits and not restricted to pharmacy benefits.
  - b. Pharmacological doses of vitamins and amino acids used specifically for the treatment of inborn errors of metabolism for those conditions recommended by the Committee under the direction of a physician will be covered.
  - c. A minimum yearly coverage should be set for all health insurance plans, including those covered by the Children’s Health Insurance Program, Tricare, and Medicaid and those governed under the ERISA. The Secretary will have authority to set age-specific minimum levels of coverage and periodically update these levels based on a standard cost of living index.

Sincerely yours,

A handwritten signature in cursive script that reads "R. Rodney Howell".

R. Rodney Howell, M.D.  
Chairperson

Enclosures:

Tab A: List of State Laws

Tab B: Table of Conditions and Medical Foods

Tab C: Review of Treatment in PKU