



## **Medical Foods Legislation**

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Mother of 2 PKU children



# Mission Statement

To improve the lives of individuals and families with PKU through research, support, education and advocacy, while ultimately seeking a cure.

# NPKUA Member Organizations

- Children's PKU Network
- Florida PKU Foundation
- Indiana PKU and Allied Disorders Association
- Intermountain PKU and Allied Disorders
- Iowa PKU Foundation
- Maryland Alliance of PKU Families
- The Michaux Family Foundation for PKU
- Michigan PKU and Allied Disorders
- The Mid-Atlantic Connection for PKU and Allied Disorders
- Minnesota PKU Foundation New England Connection for PKU and Allied Disorders
- New England Connection for PKU and Allied Disorders
- North Texas PKU Association
- PKU Pursuits
- PKU Organization of Illinois
- Ryan's PKU Foundation
- Tennessee PKU Foundation
- Wyoming PKU Foundation

# Advocacy Campaign

- Health care reform opportunity
- Kelly McDonald, Advocacy Committee chair
- Gathered Health Care Stories
- Spent the summer on The Hill



Improve our Future and  
Save Healthcare Dollars

# Educating Congress



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Secretary's Advisory Committee on Heritable  
Disorders in Newborns and Children  
5600 Fishers Lane, Room 18A19  
Rockville, Maryland 20857  
(301) 443-1080 – Phone  
(301) 480-1312 – Fax  
[www.hrsa.gov/heritabledisorderscommittee](http://www.hrsa.gov/heritabledisorderscommittee)

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

- 100 Senators
- More than 200 Reps

# Initial Success

- Senator John Kerry (D-MA) has agreed to draft legislation to federally mandate insurance companies to cover medical foods and foods modified to be low in protein for PKU and 29 other IEM's
- Dr. Rodney Howell



# Advocacy Work Continues

- Last week, more than 900 emails were sent to Members of Congress on the importance of coverage from the PKU community
- Coordinated campaign of phone calls, emails, in-district meetings, and letters to the editor
- Working in partnership with other IEM and rare disease orgs
- Looking for denials from Medicaid and/or Medicare



# Thank You!



- Your letter has made a difference for PKU families
- Used the letter as part of our talking points
- Legitimacy
- Fight ahead to advance our cause for all children and adults with IEM's





National  
**PKU**  
Alliance

research | education | support | advocacy

Together,  
we can  
change the  
history of  
PKU

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