

September 17, 2010

Access to Medical Foods and Related Products: Summary of Regional Surveys

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

Follow-up and Treatment Subcommittee

**Susan A. Berry, M.D.
For the Medical Foods Expert Panel**

Charge of the Follow-up and Treatment Subcommittee

- ▣ Identify barriers to short- and long-term follow-up of NBS results specific to the challenges in integration of health care systems, financing of services and information technology
- ▣ Develop recommendations for overcoming identified barriers in order to improve short- and long-term follow-up of NBS results
- ▣ Recommend mechanisms for establishing accountability for NBS follow-up guidelines

Why did we do this?

Don't know

Section E. This section is for any comments you may have about this survey. We welcome any comments you might wish to share You may use the back of this page.

I do not think it is fair for children with medical problems and you do not get help to provide for your child. I have a child with LCHAD & I am very short for money and unable to work do to my child's needs. I am getting help on medical bills but not for her MCT oil that she needs for the rest of her life or she may not survive. I currently have that medical in collection cuz I am unable to pay for. What I am suppose to do to pay for it and keep getting her meds. Also it is very expensive to buy the foods she requires. I do not get help for her fat free - low fat foods. Please help pass this on thru states and put yourself in our position.
(What do u do?)

What is the problem?

- Medical foods, often referred to by families as “special formulas” or “protein substitutes” are NOT drugs, they are substances of nutritional value
- Medical foods are not optional, they ARE the treatment
- Treatment is LIFE-LONG
- Everyone needs food, yet traditional foods can be harmful to persons with inborn errors of metabolism
- Medical foods are substantially more expensive than traditional foods
- Because they are foods, they are EXCLUDED from coverage by many insurers
- Costs may be prohibitive; coverage is at best variable
- *Affected persons cannot survive without medical foods but they cannot afford to buy them*

FDA Definition: Medical Food

“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

(Is the definition part of the problem?)

What are the nutritional treatments?

- Medical foods
 - ▣ Specially compounded formulas that supply a substantial portion of nutrition for the treatment of inborn errors of metabolism
- Supplements or “Nutriceuticals”
 - ▣ Pharmacologic doses of cofactors or vitamins, e.g. biotin for biotinidase deficiency
 - ▣ Amino acids provided to give substrate or prevent specific amino acid deficiency
 - ▣ Other vitamin-like drugs that may provide benefit, e.g. carnitine
 - ▣ MCT oil
- Specially manufactured modified low-protein foods (MLPF)

Medical foods require physician supervision

All are essential elements of therapies for treatment of inborn errors of metabolism

Medical equipment and supplies may be needed for feeding

Follow-up and Treatment Subcommittee Addresses Challenges

- Medical Foods Workgroup meeting, June 2, 2008: Insurance coverage of medical foods, modified low protein foods, nutritional supplements/nutriceuticals and related feeding supplies – private and public insurance perspectives, and employment-based health plan issues
- Medical Foods Survey: Parent Survey of Insurance Coverage of Medical Foods for Children with Metabolic Conditions

What are (some of) the barriers?

- Each insurer has its own practices
 - ▣ Private insurers (public using private vendor, self-insured, employers)
 - ▣ Public practices vary state to state
- Each policy, even with the same company, may have differing coverage; contracts may result in differences for the SAME insurer
- Each state has different rules / laws covering provision of medical foods
 - (see <http://www.ncsl.org/programs/health/lawsfoodsformula.htm> for list of laws)
- Even when laws exist they may not cover all insurance carriers (ERISA)
- Even when laws/guidelines exist they are subject to interpretation by insurers and the states

Follow-up and Treatment Subcommittee Addresses Challenges

- Medical Foods Workgroup meeting:
Insurance coverage of medical foods, modified low protein foods, nutritional supplements/nutriceuticals and related feeding supplies – private and public insurance perspectives, and employment-based health plan issues
- Medical Foods Survey: Parent Survey of Insurance Coverage of Medical Foods for Children with Metabolic Conditions, 2008-2010

Objectives of Medical Foods Survey

- Purpose: to survey parents of children (birth to 18 years of age) with metabolic disorders to assess current health insurance coverage and actual coverage for medical foods (and related materials).
- 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.
- Information sought:
 - ▣ 1) What are the needs of children for metabolic foods and formulas, modified low protein foods, prescribed dietary supplements, and medical feeding supplies and equipment?
 - ▣ 2) What are the out-of-pocket expenses for foods and formulas?
 - ▣ 3) What proportion of expenses for medical foods is paid for?

Preparation of Medical Foods Survey

- Expert panel established: metabolic genetic physicians, metabolic nutritionist, epidemiologist, statistician, parent, public health professionals.
- Parent Group cognitive interviews
 - ▣ Westchester Medical Center, NY: October 2007
 - ▣ Mount Sinai Medical Center, NY: March 2008
- Pre-testing Survey validity/reliability, Fall 2008
 - ▣ Emory University
 - ▣ University of Minnesota
 - ▣ University of Pittsburgh

Content

The survey asked families about:

- Their child's diagnosis
- The health plan(s) covering the child's care
- The medical foods/formulas, modified low protein foods, prescribed dietary supplements, and medical feeding supplies/equipment used by their child
- The extent to which those items were covered by their health plan, including dollar amounts per month
- Their monthly out-of-pocket expenses for those items, if not fully covered
- If their health plan had caps on the coverage that was provided

Conducting the Survey

- Implementation by HRSA Regional Genetics and NBS Services Collaboratives 2 (NYMAC), 3 (SERC) and Region 4 with support from the National Newborn Screening and Genetics Resource Center (NNSGRC): late Spring 2009
- Targeted at families with children (0-18 years) with metabolic disorders treated with medical foods, supplements, modified low-protein foods; also asked about feeding supplies
- 305 families responded across all three regions.

Survey Implementation: Genetic Centers

- IRB Process – approval received for implementation
- Paper survey administered by genetic centers
- Parent Responders completed survey anonymously
- Limited demographics collected –
 - ▣ State of residence
 - ▣ Age of child
 - ▣ Diagnosis
- Genetic Centers submitted data to their Regional Collaborative (RC)
- Each RC used data to inform their own planning
- RCs submitted data to HRSA MCHB for integration

Acknowledgment: Genetics Centers

Region 4:

University of Minnesota Amplatz
Children's Hospital
Cincinnati Children's Hospital
Medical Center

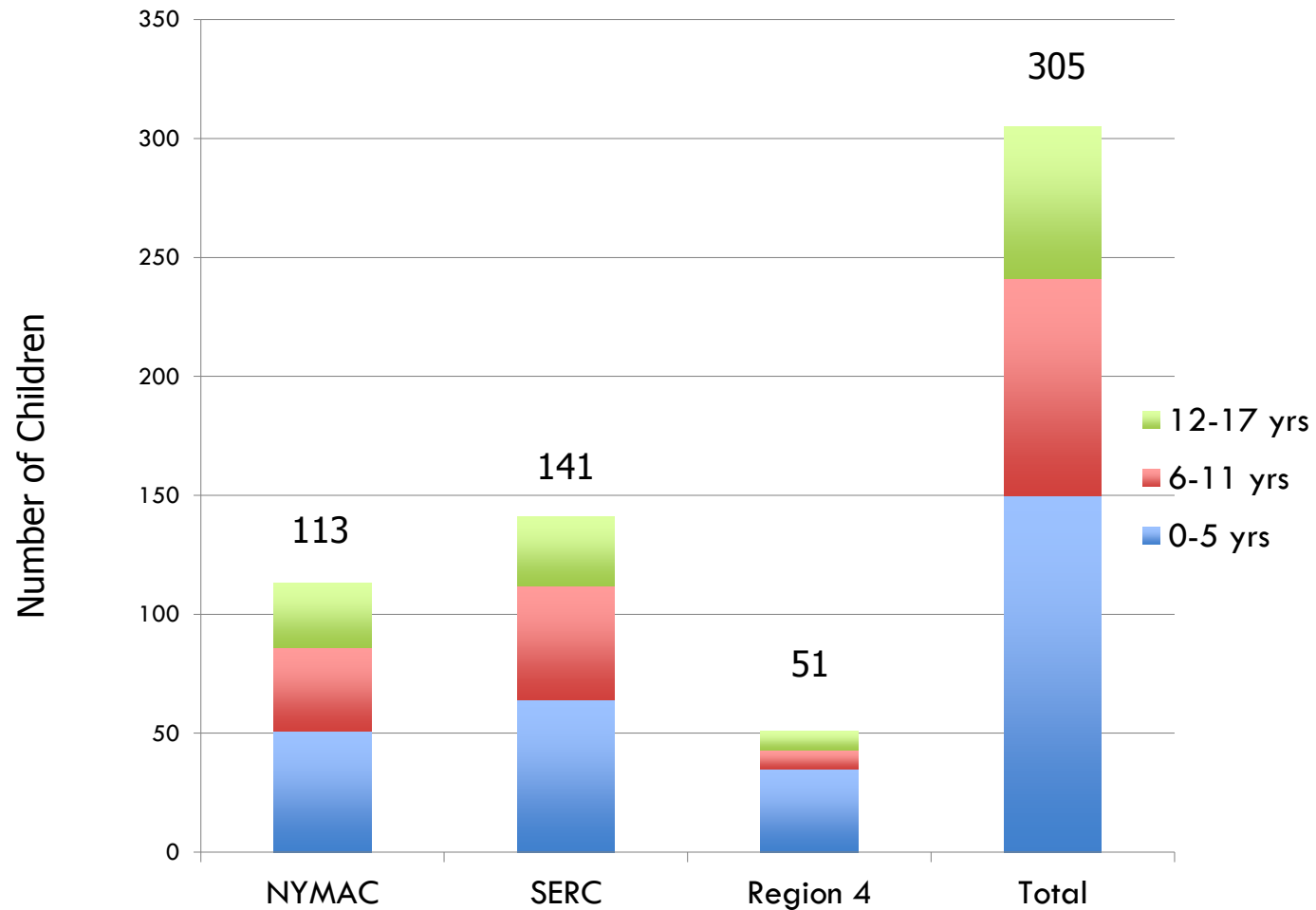
SERC:

Emory University
Greenwood Genetics Center
University of Florida
University of North Carolina -
Chapel Hill
University of Tennessee
Vanderbilt University Medical
Center

NYMAC:

Mount Sinai Medical Center
Children's Hospital at Albany
Medical Center
Children's Hospital of Pittsburgh
Golisano Children's Hospital at
Strong
Maria Fareri Children's Hospital
at Westchester Medical Center
University of Maryland Hospital
for Children

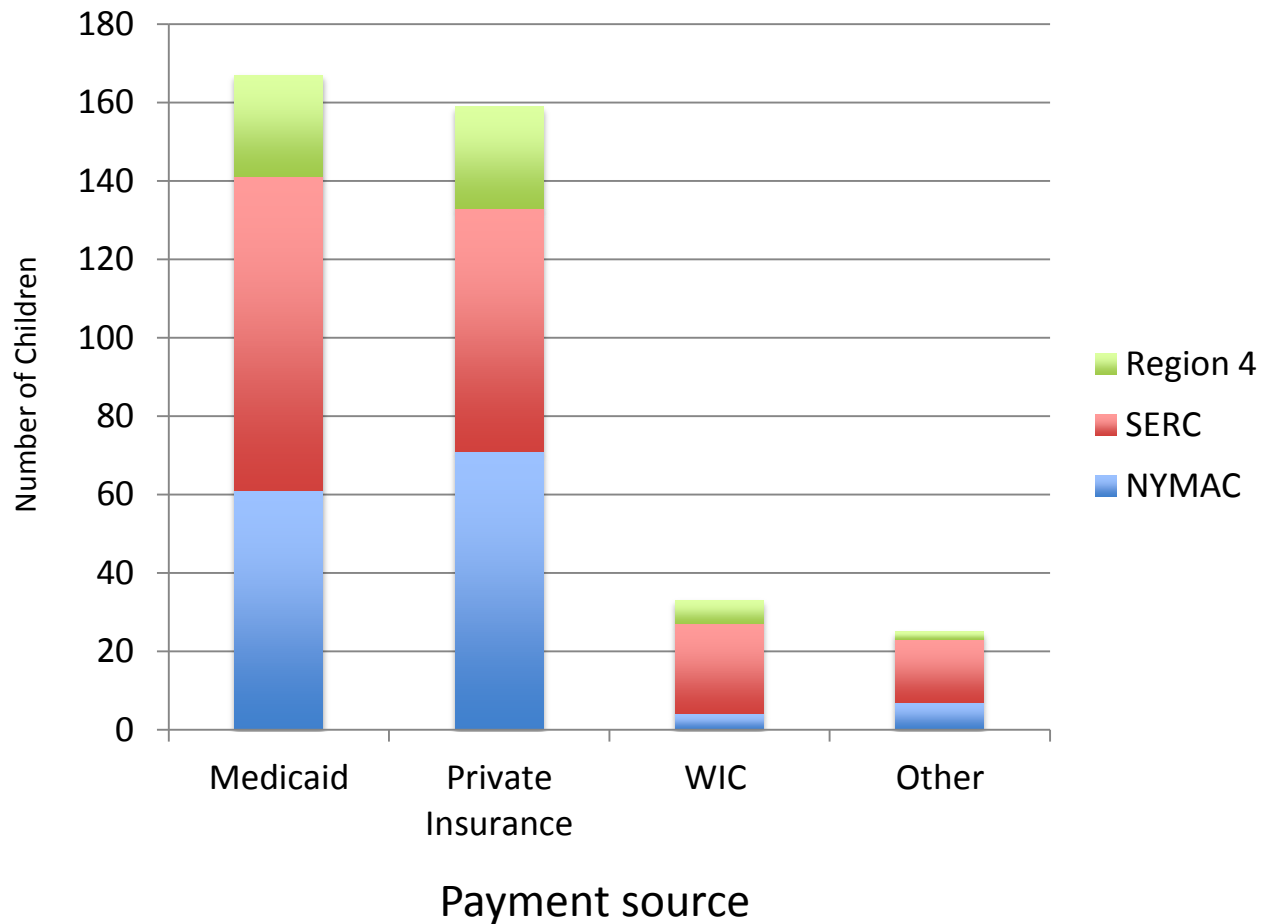
Ages of Children Surveyed



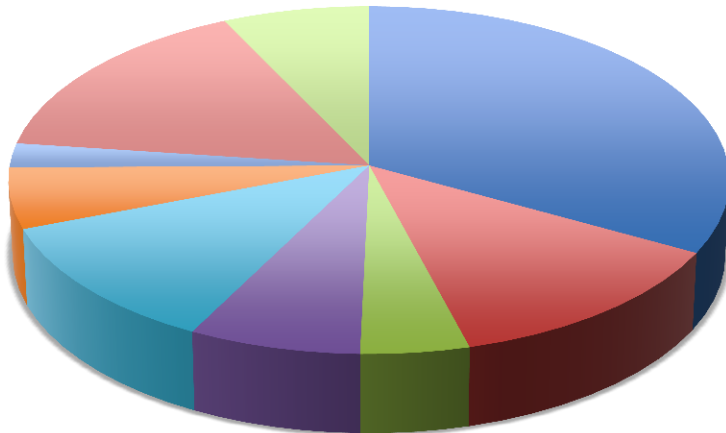
Health Care Coverage

25% of children had more than one funding source

Only 3 children had NO coverage

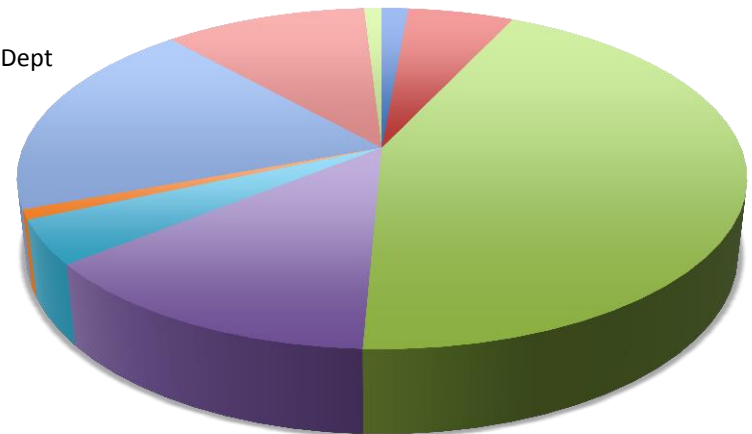


Sources of products

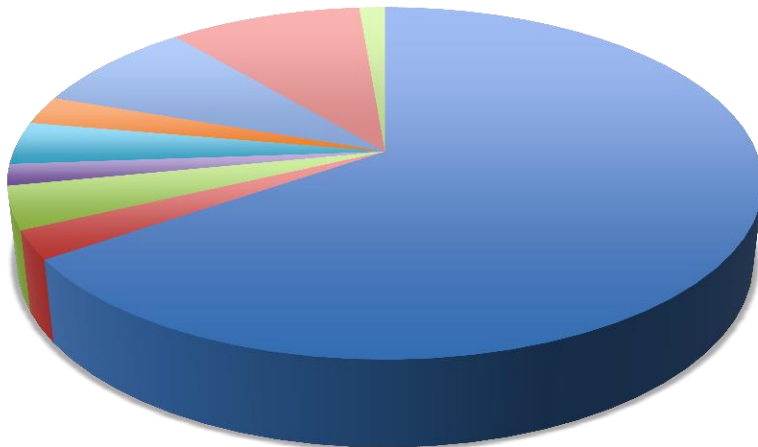


Medical Foods

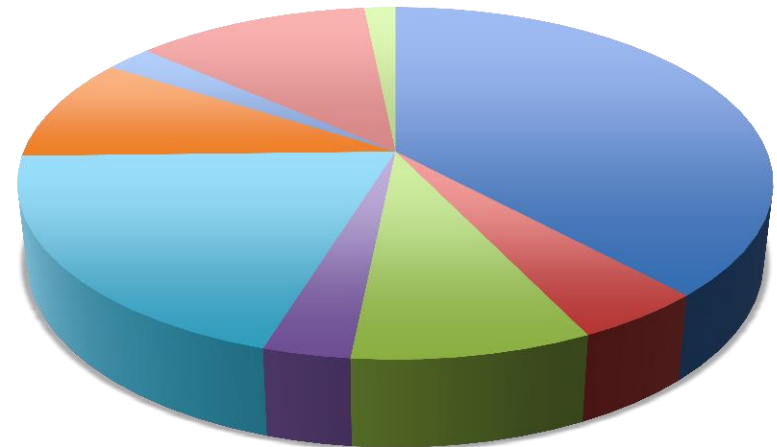
- Pharmacy
- StateCountyHealthDept
- Internet
- Manufacturer
- MedicalSupplyCo
- HomeHealthCo
- HealthFoodStore
- HospitalOrClinic
- WIC
- Other



Modified Low-Protein Foods (MLPF)



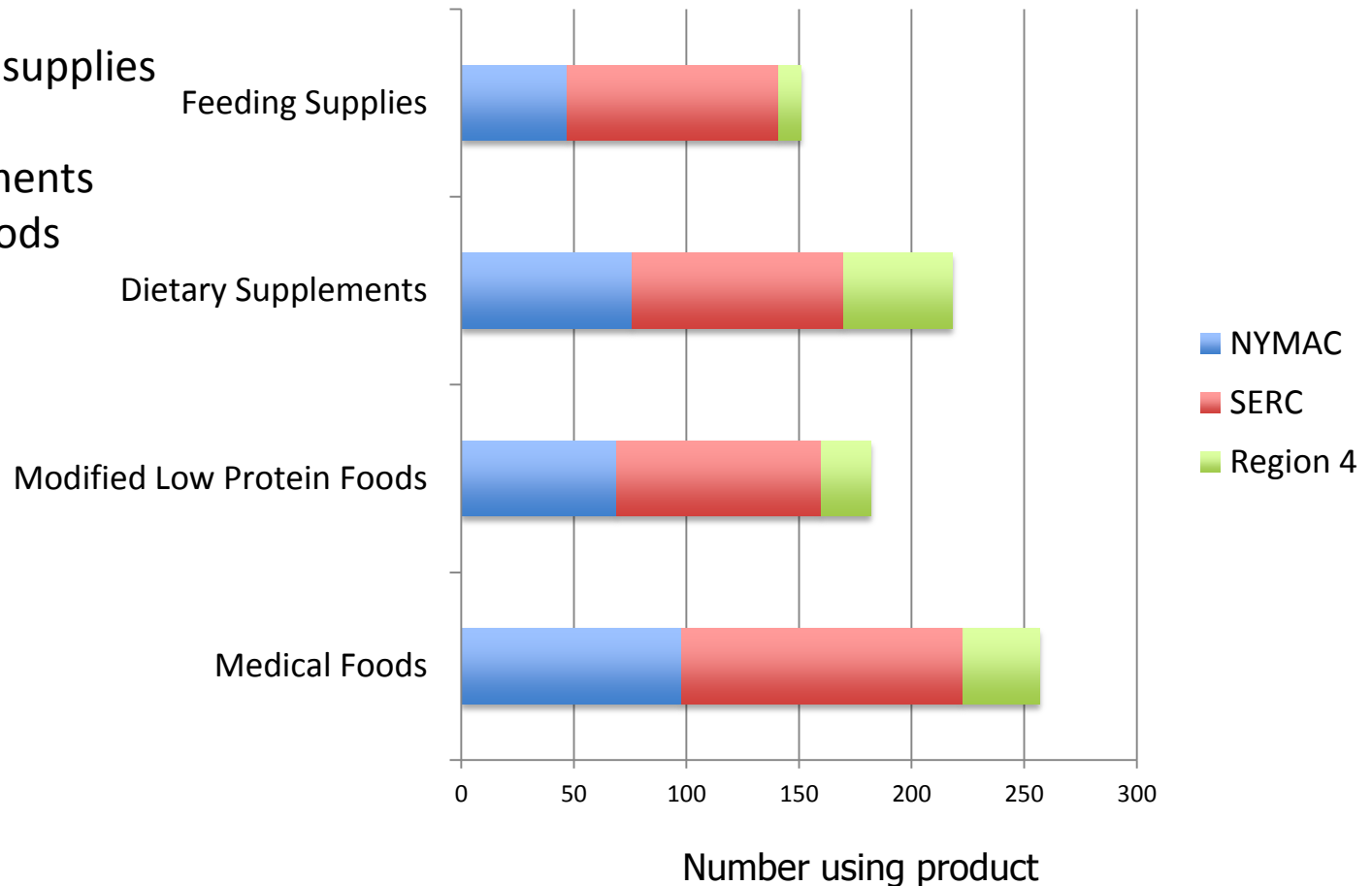
Dietary Supplements



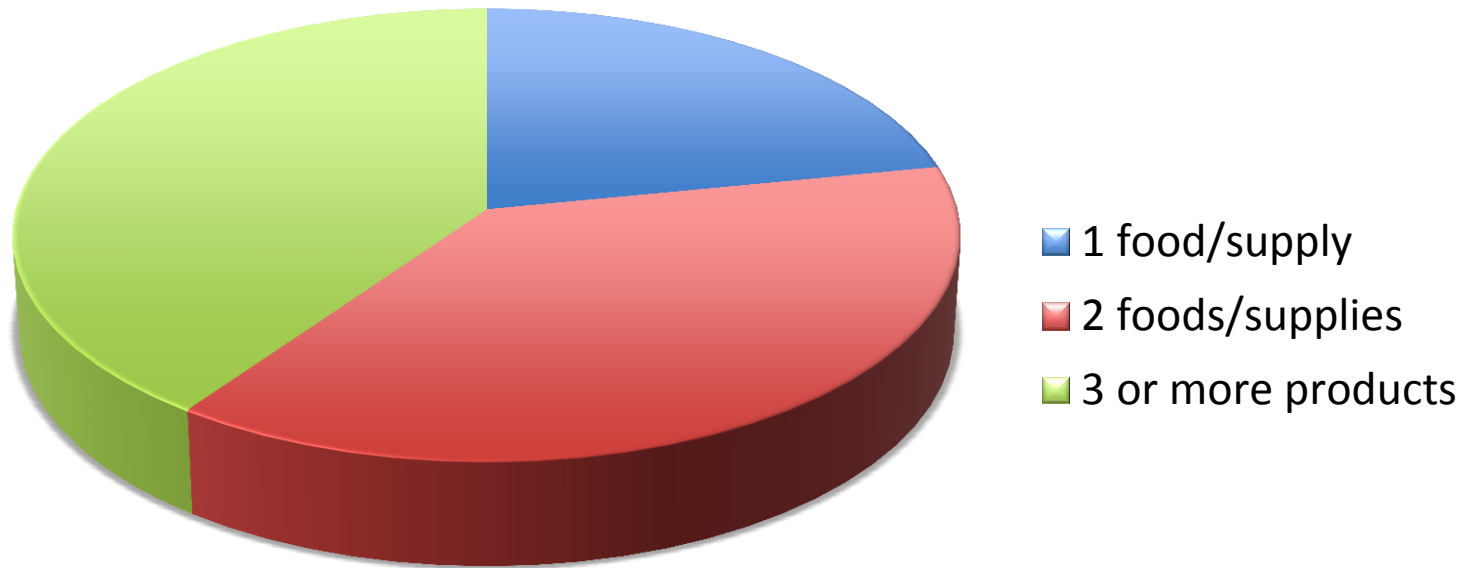
Feeding Supplies

Children need lots of products for nutritional support of their conditions

50% used feeding supplies
60% used MLPF
71% used supplements
84% used Med Foods



Most children use more than one category of product

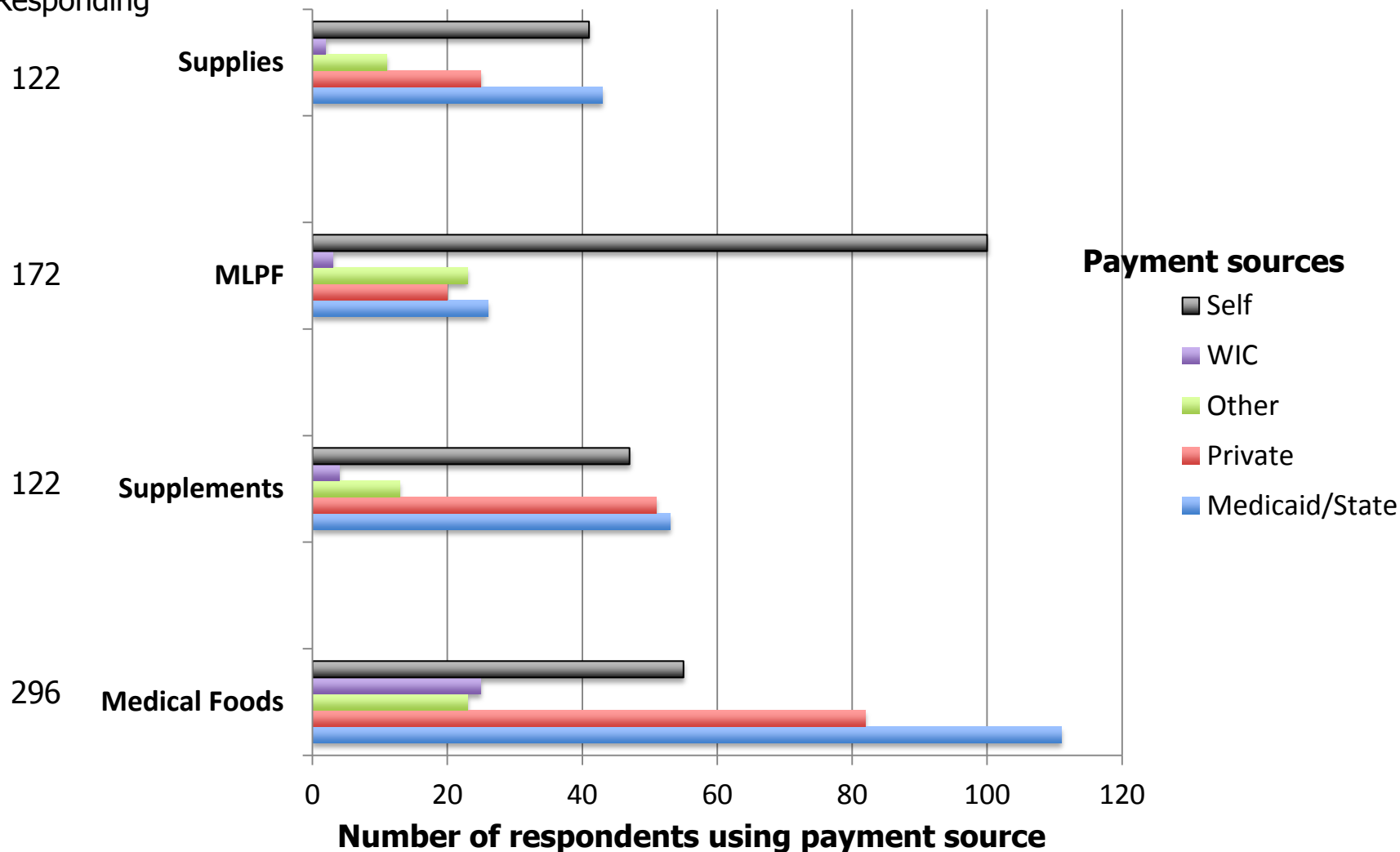


Percentage of children using products on a daily basis

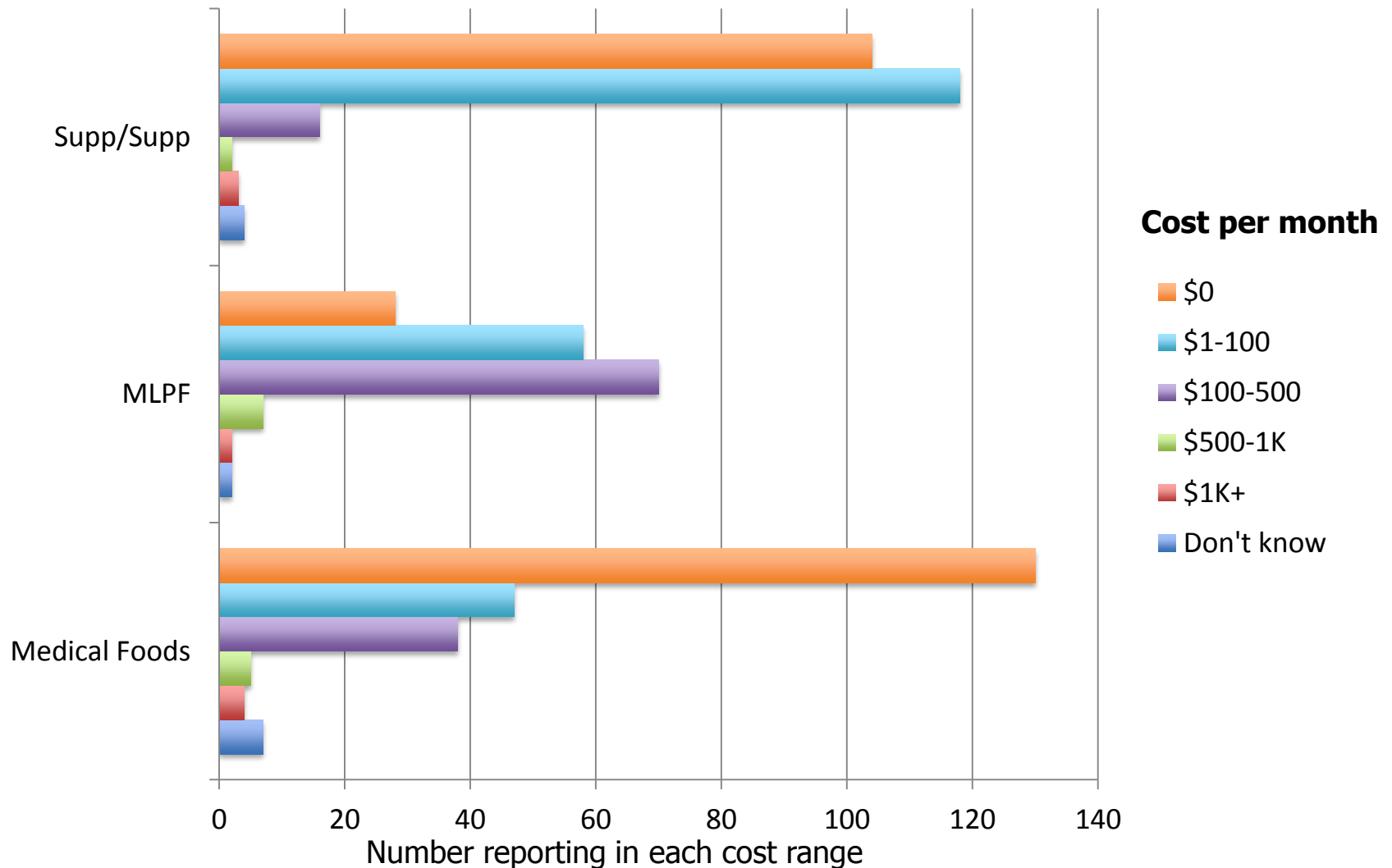
80% use at least 2 of the surveyed products

Families pay out-of-pocket for all types of products, especially MLPF

Total Responding



Although many pay little or nothing, some families pay substantially



What did we learn?

- Nearly all children in this group had some type of health care coverage (even if it did not pay for these products)
- Most children needed more than one category of food/supplies
- 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

What we do and don't know...

- Families often did not know if they had “caps” on insurance and what the dollar amount of the cap was
- Families had a hard time telling us their out-of-pocket costs (so 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 very important 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*

What has happened?

- SACHDNC has communicated three times with the Secretary of HHS regarding Medical Foods
 - SACHDNC Letter of April 7, 2009: Recommendations for Medical Foods
 - Interim Response: [“beyond the Department’s authority...I am neither adopting nor rejecting the Committee’s recommendations”]
 - SACHDNC Letter of March 23, 2010: Gaps in Insurance Coverage
 - Interim Response --- [“...A thorough response to this letter will be forthcoming upon a careful review of the report...” response due September, 2010]
 - SACHDNC Letter of June 14, 2010: Recommendations for Medical Foods in light of health care reform
 - Response --- [interim response received July 29, 2010: response due December 2010]

Medical Foods Equity Act

- House of Representatives bill HR4926: 27 co-sponsors
- Senate bill S2766: 2 co-sponsors
 - Addresses coverage of medical foods for life
 - Based on SACHDNC recommendations
 - Endorsed by over 40 organizations
 - Covers 29 Inborn Errors of Metabolism (IEMs)
 - Requires coverage by all private insurance plans and federal health programs
 - Health insurance plans governed by ERISA would not be exempt
 - HHS would determine the minimum yearly coverage for all health insurance plans
 - Would not pre-empt state standards that require a higher minimum standard

SACHDNC - What next?

- Anticipate response(s) from the HHS Secretary
- Monitor progress of the Medical Foods Equity Act and Benefits Package for Affordable Health Care Act
- Work with FDA (Orphan Products, Center for Food Safety and Nutrition)
- For the Subcommittee:
 - ▣ Extend the survey (other regions, other ages)?
 - ▣ Focus on elements of highest impact?
 - ▣ Publication?

We stand prepared to move this further!