

## **Insurance coverage of medical foods for treatment of inherited metabolic disorders**

**Running Title:** Medical food insurance coverage

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**ABSTRACT**

*Purpose:* Treatment of inherited metabolic disorders is accomplished by use of specialized diets employing medical foods and medically necessary supplements. Families seeking insurance coverage for these products express concern that coverage is often limited; the extent of this challenge is not well defined.

*Methods:* To learn about limitations in insurance coverage, parents of 305 children with inherited metabolic disorders completed a paper survey providing information about their use of medical foods, modified low-protein foods, prescribed dietary supplements, and medical feeding equipment and supplies for treatment of their child's disorder as well as details about payment sources for these products.

*Results:* Although nearly all children with inherited metabolic disorders had medical coverage of some type, families paid "out-of-pocket" for all types of products. Uncovered spending was reported for 11% of families purchasing medical foods, 26% purchasing supplements, 33% of those needing medical feeding supplies, and 59% of families requiring modified low-protein foods. Forty-two percent (42%) of families using modified low-protein foods and 21% of families using medical foods reported additional treatment-related expenses of \$100 or more per month for these products.

*Conclusion:* Costs of medical foods used to treat inherited metabolic disorders are not completely covered by insurance or other resources. (200 words)

**Keywords:** Inborn errors of metabolism, insurance coverage, neonatal screening, diet therapy

**ABBREVIATIONS**

IMD - inherited metabolic disorders  
HRSA - Health Resources and Services Administration  
MCHB - Maternal and Child Health Bureau  
FDA - Food and Drug Administration  
WIC - Women, Infants and Children  
ERISA - Employee Retirement Income Security Act  
OOP – “out-of-pocket”

## INTRODUCTION

Inherited metabolic disorders (IMD) are a complex group of genetic conditions where an enzyme deficiency prevents normal metabolism. When the enzyme deficiency prevents normal use of nutrients, effective treatment of the associated IMD depends primarily on dietary restriction, since use of off-the-shelf foods containing substances that cannot be metabolized by the affected person results in toxic effects. Typically, a healthcare provider prescribes a specialized diet restricting the food component that cannot be metabolized. For example, individuals affected with phenylketonuria are unable to properly metabolize the amino acid phenylalanine, which must be selectively limited in their diet to prevent severe intellectual disability. For some disorders, food products restricted in the offending material are available without modification of conventional products, for example, galactosemia is treated in infancy by use of conventional formulas using sucrose as a sugar source rather than the galactose-containing disaccharide, lactose. For others, particularly disorders of amino acid metabolism, conventional products that restrict the offending amino acid(s) are not available. Treatment of disorders of amino acid metabolism requires products specially manufactured to exclude an offending amino acid or amino acids. These are typically supplied in powdered formula or in other product forms such as bars, capsules, or ready to consume liquids. Beyond powdered formulas, bars and capsules, physicians can also prescribe modified low-protein foods: products specially manufactured to provide versions of common foods made with restricted protein content. Such preparations are referred to collectively as “medical foods,” defined by the Food and Drug Administration (FDA) in section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3))

as “food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for specific dietary management of the disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.”<sup>1</sup>

In addition to medical foods (formulas, bars, and modified low-protein foods), other specialized nutritional products, including high doses of vitamins and amino acids, are used in the management of IMD. The use of medical foods, modified low-protein foods, amino acid supplements, and high doses of vitamins for individuals with IMD is not merely an optional, alternative food choice, but rather a medical necessity.

Although each metabolic disorder has its own pathological profile if left untreated, children who cannot consistently obtain specialized nutritional products may experience serious adverse health effects, including severe acidosis and/or coma requiring complex hospital care, intellectual impairment, behavioral dysfunction, inadequate growth, abnormal development, nutrient deficiencies, and even death.

The cost of these foods and products can be high. The prepared formulas that comprise the major part of the medical foods used for IMD treatment are more expensive than standard infant formulas. For example, a case of standard powdered infant formula costs the state of Mississippi Women, Infants and Children (WIC) program \$17.66, while a case of a specialized medical food formula for infants costs \$202.32. A case of formula designed for an older child costs \$405.60.<sup>2</sup> Modified low-protein foods are also much more expensive than corresponding non-modified products. A box of low-protein

chocolate cake mix costs \$10 compared with \$3.25 for a generic store brand of cake mix. A box of low-protein pasta costs \$9.25, nearly four times the price of regular store brand (\$2.49).<sup>3</sup> Overall, modified low-protein foods cost two to eight times more than their unmodified counterparts.<sup>4</sup>

Some states pay for and provide medical foods as a part of their overall Newborn Screening (NBS) program. Most states that do not directly provide medical foods have enacted legislation that requires insurers to provide coverage for medical foods.<sup>5,6</sup> However, policies regarding coverage vary from state to state and even when legislation has been enacted, there are exceptions and exclusions, such as the Employee Retirement Income Security Act (ERISA) exemptions to state legislative mandates.<sup>6</sup> Since levels of insurance coverage for medical foods are not uniform across states,<sup>7</sup> three of the Regional Genetic and Newborn Screening Service Collaboratives surveyed families with a child affected with an IMD requiring medical foods, modified low-protein foods, high doses of vitamins, and/or amino acid supplements. The purpose of the survey was to ascertain use of these products, sources and methods of payment, limitations in insurance coverage for purchase of the products, and the amount of “out-of-pocket” (OOP) expenditures required of the surveyed families to make these medically necessary purchases.

## **METHODS**

### **Survey Development**

Working jointly, three Health Resources and Services Administration (HRSA)

Maternal and Child Health Bureau (MCHB) Regional Genetic and Newborn Screening Services Collaboratives (<http://www.nccrcg.org>): Region 2 New York – Mid-Atlantic Consortium for Genetic and Newborn Screening Services (NYMAC; includes Delaware, District of Columbia, Maryland, New Jersey, New York, Pennsylvania, Virginia and West Virginia); Region 3 Southeast Newborn Screening and Genetics Collaborative (SERC; includes Alabama, Florida, Georgia, Louisiana, Mississippi, North Carolina, Puerto Rico, South Carolina, Tennessee, Virgin Islands); and Region 4 Genetics Collaborative (comprising Illinois, Indiana, Kentucky, Michigan, Minnesota, Ohio, and Wisconsin) developed a survey instrument and conducted the survey. The survey was piloted in two centers in different Regions and two family focus groups provided feedback regarding the instrument. Questions were refined based on feedback from the focus groups and results of the survey pilot.

### **Survey Administration**

Materials for consent were prepared and Institutional Review Board approvals obtained for administration of a pen and paper survey based on a convenience sample of parents of children with an IMD. Recruitment methods varied by clinic site, but in general all parents of children with conditions requiring the use of medical foods that had clinic appointments during the summer of 2009 were asked to participate. Parents of 305 children less than 18 years of age completed the anonymous survey during regular clinic visits. The survey was administered in 14 centers in the three participating regions. Twenty-two of the respondents lived in one of the nine states where medical foods are supplied as part of their state's newborn screening program (North Carolina).<sup>5</sup>

Medical diagnosis and medical food reference lists were included with the survey document as aids for parents in responding to survey questions.

### **Data and Survey Analysis**

The final paper survey included questions about health insurance coverage for the child's health care (i.e., public or private insurance, WIC, military/veterans program, Indian Health Service, or other source); the medical foods, modified low-protein foods (e.g., low protein pastas, breads, cereals, etc.), prescribed dietary supplements (i.e., amino acids, vitamins, carnitine or other supplements), and medical feeding supplies/equipment (e.g., pumps, port-o-caths, syringes, tubing, Mic-key buttons, etc.) used by the child; an estimate of how much families had to pay OOP for their child's dietary treatment (asked by ranges; \$0/month, >\$0 to <\$100/month, \$100 to <\$500/month, \$500 to \$1,000/month, and >\$1,000/month); the extent to which these items were covered by their health plan; and the annual "cap" or limit on the amount covered by the family's policy. Demographic information was limited to age, diagnosis, and state in which the child resided. Data on annual caps are not reported here due to the number of null responses.

The data were first analyzed using basic descriptive statistics such as frequencies and percentage statistics for the survey items described above. Chi square tests were used to determine if there were statistically significant differences in the distribution of 1) reported payment sources used by parents purchasing medical foods, modified low protein foods, dietary supplements, and medical feeding supplies and 2) the level of

OOP expenses families paid for the four different product types. The units of analysis for these tests were mentions of reported payment sources. Bonferonni-adjusted post-hoc tests ( $p=0.005$ ) were performed following the finding of significant results for each chi square test.

**Results:**

Of the 305 children of families that responded to the survey, 150 (49%) were newborn to five years of age, 91 (30%) were between six and 11 years, and 64 (21%) were between 12 and 17 years. Fifty percent of the children had phenylketonuria, 10% had other amino acidemias, 12% had organic acidemias, 6% had urea cycle disorders, 12% had fatty acid oxidation disorders and 10% had other conditions or an unspecified diagnosis. The distribution of disorders reflects the general prevalence of the disorders within the U.S. population and the nature of therapies for the conditions.

The survey asked families to describe all sources of insurance coverage [private, public and none] for healthcare or payment through WIC for medical foods and formula available to them. In this sample, only three families specified that their children had no coverage for healthcare. Twenty-five percent of the children had multiple funding sources for their coverage. Medicaid (55%, 167 families) and private insurance (52%, 159 families) were common sources of partial or full coverage reported by families. WIC was the only other entity reported as a source of coverage by more than 10% (33) of families. Of the subset of 22 patients from a state where medical foods are provided by the NBS program, multiple sources were still employed and only one family

indicated they received their medical foods from the state program.

Families reported obtaining their dietary products, supplements and supplies from a variety of sources, depending on the nature of the product being used. Families needing medical food reported purchasing them primarily from pharmacies (34%), hospital/clinics (18%), health departments (14%), and medical supply companies (12%). Over two-thirds (69%) of families needing dietary supplements reported pharmacies as the source from which they obtained these products. In contrast, families requiring modified low-protein foods largely obtained them from Internet sources (41%), health food stores (16%), and directly from manufacturers (14%). Among families purchasing feeding supplies, pharmacies (32%), medical supply companies (17%), and clinics (18%), were the principal sources. For each product type, however, families often obtained the products from multiple sources.

Affected children often required items from multiple categories for nutritional support of their conditions – 257 families (84%) reported using medical foods, 181 families (59%) reported using modified low-protein foods, 152 families (50%) reported using one or more dietary supplements and 152 families (50%) reported using feeding supplies. Overall, the parents surveyed indicated that 80% of children used at least two of the surveyed product types and 48% used three or more.

Families relied on multiple payment sources for individual products and/or may have used multiple payment sources for multiple product types. Figure 1 shows all reported

payment sources by product type. Chi square test results indicated that across the product types there were significant differences in the distribution of reported payment sources used to purchase the different types of products ( $\chi^2 = (12, N=753) 159.53$ ,  $p < 0.005$ ). Medicaid often covered medical foods, dietary supplements, and feeding (40%, 35%, and 31% of reported sources, respectively). Private insurance also frequently contributed to the coverage of medical foods (30%) and dietary supplements (32%).

Of note was the frequency of OOP expenses parents were required to pay for nutritional treatment of their affected child due to a lack of coverage from one of the other sources included in the survey (i.e., Medicaid, private insurance, WIC, or other source).

Families paid OOP for all types of products, most notably for modified low-protein foods. OOP spending accounted for 11% of medical foods payment sources, 26% of supplement sources, 33% of feeding supplies payment sources and approximately 60% of payment sources for modified low-protein foods. Bonferroni-adjusted post-hoc tests indicated that modified low-protein foods were the only product type for which the percentage of total mentions of outside coverage was not more frequent than OOP purchasing (medical foods:  $\chi^2 (1, N=281) = 170.08$ ,  $p < 0.005$ ; modified low-protein food:  $\chi^2 (1, N=174) = 5.89$ ,  $p > 0.005$ ; dietary supplements:  $\chi^2 (1, N=160) = 36.1$ ,  $p < 0.005$ ; and medical feeding supplies  $\chi^2 (1, N=138) = 16.4$ ,  $p < 0.005$ ).

Figure 2 shows the percentage of parents reporting OOP expenditures by product type. Chi square test results indicated that across product types there were statistically

significant differences in the distribution of OOP expenditure levels for the different types of products ( $\chi^2$  (9, N=632) = 141.27,  $p < 0.005$ ). Twenty-one percent (21%) of parents purchasing medical foods paid more than \$100 per month (beyond the costs for an unaffected child) as did 48% of parents purchasing modified low protein foods, 11% purchasing dietary supplements, and 6% requiring feeding supplies. Some families reported paying \$500 or more for medical foods (4%), modified low protein foods (5%), dietary supplements (2%) and feeding supplies (3%). Bonferroni-adjusted post-hoc tests indicated that modified low-protein foods were the only product type for which parents overall reported paying in excess of \$100/month more often than they paid less than \$100/month (medical foods:  $\chi^2$  (1, N=224) = 59.93,  $p < 0.005$ ; modified low-protein food:  $\chi^2$  (1, N=165) = 0.30,  $p > 0.005$ ; dietary supplements:  $\chi^2$  (1, N=123) = 73.37,  $p < 0.005$ ; and medical feeding supplies  $\chi^2$  (1, N=120) = 93.6,  $p < 0.005$ ). Though the majority of parents purchased multiple product types to fully meet the medical needs of their children, we were not able to determine the additive OOP costs for those parents due to the categorical nature of the response options.

Overall, the survey results found that 41% of families had OOP expenses for some of the products that were purchased to maintain IMD treatment with some families paying for the cost of multiple products. Medical foods were more frequently covered by insurance and obtained from medically based sources, while families more often paid out-of-pocket for modified low-protein foods and obtained these from the Internet or from the manufacturer directly.

**DISCUSSION:**

This is the first survey to obtain specific information about insurance coverage available to families who must use medical foods and related products. The populations of the three Regions in this collaboration represent 57% of the US population, based on U.S. 2010 Census figures. Though each state has a different regulatory environment for payment for these products, when we summarized the data from these centers our findings reveal at best uneven coverage for these needed treatments with an associated potential for financial burdens for these families managing their child's chronic metabolic disorder.

We found that some families experienced additional expenditures to cover the costs of their child's required medical food treatment. Many had OOP expenses; a few families had expenses amounting to more than \$500 per month. Although 99% percent of the children in our survey had some type of healthcare coverage, the insurance often did not provide complete coverage for medical foods, modified low protein foods, supplements or feeding supplies necessary for management of IMD.

Notably, the level of health insurance coverage observed in this study is not typical of the general population, where approximately 10% of children remain uncovered.<sup>8</sup> Some of this discrepancy in coverage rates may have resulted from a selection bias from our convenience survey of individuals who already had access to health services due to their child's diagnosis. Consequently, we suggest that the families in this sample represent a best-case scenario relative to insurance coverage relative to medical foods.

Our study had specific limitations. The families as respondents had a great deal of difficulty identifying all of their OOP costs. While this underscores the complexities in understanding insurance coverage for families even when they are frequent users of this service, it also limited our ability to gather detailed quantitative information about the magnitude of expenses related to the use of medical foods and associated products. Despite these shortcomings, our observations show that families had significant OOP expenses for treatments that are essential for these complex disorders.

We also found that families depended on need-based resources, such as Medicaid or WIC, for purchasing medical foods. In several states surveyed here, WIC was an important source of support for many families. WIC programs typically provide families with formulas, including medical foods for infants and very young children. When assessed by age group, WIC provided a notable portion of required nutritional treatments for infants and younger children. However, because such programs typically terminate coverage at age five older children were unlikely to have access to this resource.

We did not extend our survey to include adults. As originally conceived, the study was undertaken with funding specific to assessment of children. However, in our observation, limitations on coverage for adults are substantially worse than that for children, particularly because resources such as WIC are not available to older children or adults. Insurance companies have also often terminated coverage for these

specialized products at age 21. Medicare Part D, a common resource for adults, excludes coverage of amino acid supplements, for example. Because the care for these conditions does not end with childhood, these exceptions and terminations of coverage are particularly disturbing.

We asked families about the sources of their products with the anticipation that if they needed to acquire products directly from the manufacturer or from web-based sources on the Internet that they were likely purchasing their products directly and paying themselves. We surmised that products obtained from a pharmacy or directly from a department of health source were likely covered, with less financial impact on the family. The reported sources of products reflected these assumptions with most medical foods, supplements, and supplies being obtained from medically based resources (pharmacies, hospital/clinic, medical supply and home health company or health department) while modified low-protein foods indeed were often obtained from external sources (Internet and manufacturer). The relation of cost of product and source of product was not ascertained by the survey and should be the subject of further investigation.

## **CONCLUSION**

As a society, we have accepted the utility of diagnosis and management of IMD by initiating newborn screening programs. The rationale for newborn screening for IMD is based on overwhelming evidence showing that initiation of prompt and effective therapies offers children with these metabolic conditions improved outcomes. For IMD

requiring special foods or formulas, dietary therapy is the primary effective treatment.

We found that insurance or other resources do not consistently cover costs of medical foods used to treat IMD, potentially resulting in inequities in access to these essential products. Greater awareness by health care providers and policy makers of the challenges due to the lack of uniform coverage might help to close these gaps.

(3002 words excluding title materials, abstract, legends, references)

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## FIGURE LEGENDS

**Figure 1:** Percentage of reported payment sources used for medical foods and related products.

Percentage of payment sources used for medical foods, dietary supplements, modified low protein foods, and feeding supplies is noted on the Y-axis. Families used more than one payment source for some products and often paid for multiple products. Self = Parents' out-of-pocket expenditures, Other = Military health benefits and miscellaneous other sources, WIC = Women, Infant, and Children Programs

**Figure 2:** Cost per month paid out-of-pocket for medical foods and related products.

Percentage of families reporting OOP expenditures for medical foods, dietary supplements, modified low protein foods, and feeding supplies is noted on the Y-axis. Families often paid for more than one product type. Families reported the range of costs they paid per month beyond insurance or other coverage for medical foods, modified low-protein foods (MLPF), supplements, and supplies. Shown here are the ranges of OOP expenditures for each type of product (on the X-axis); OOP = Out-of-pocket

Figure 1

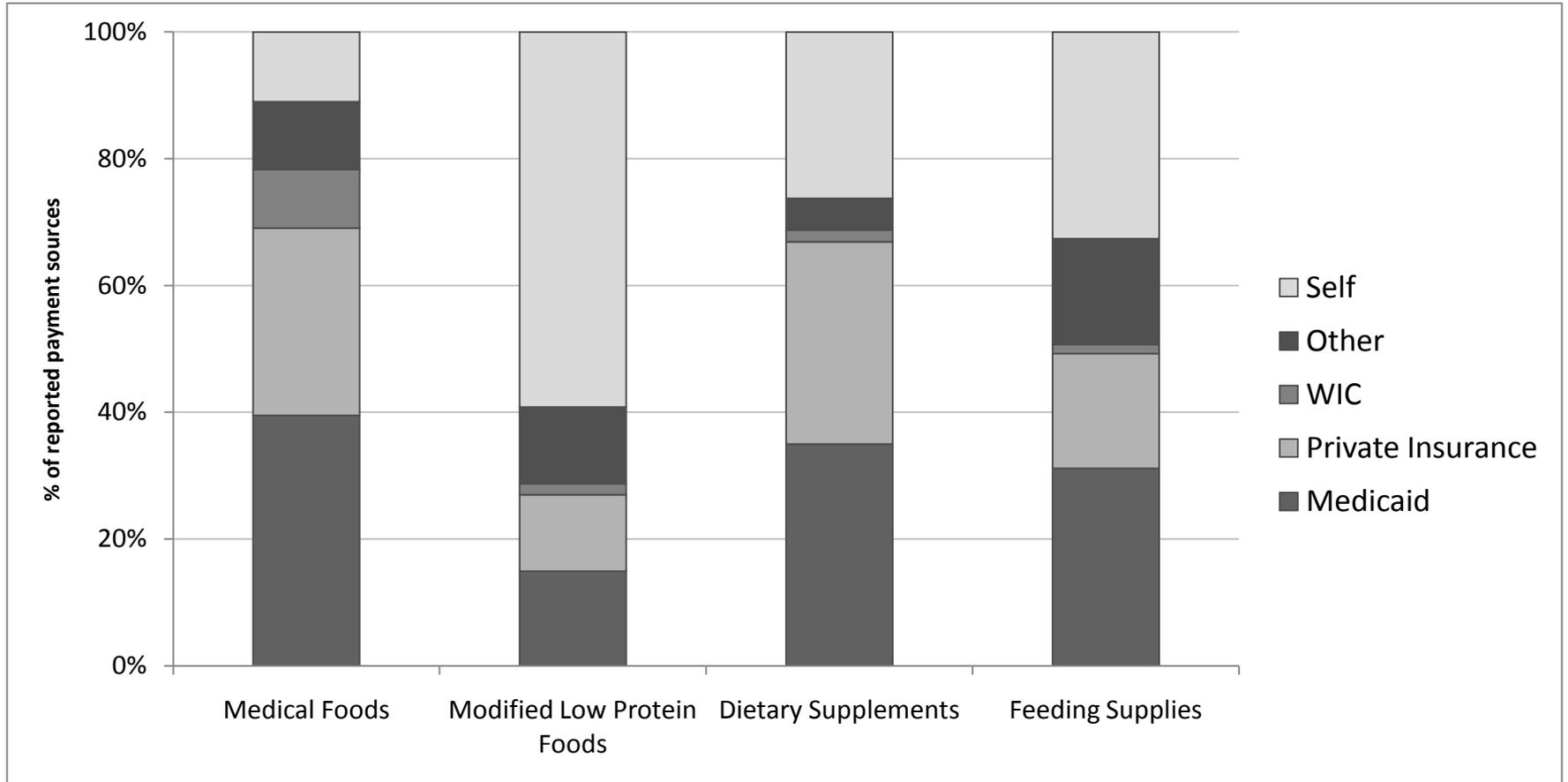


Figure 2

