
Healthcare Coverage for Medical Food Treatment of Inborn Errors of Metabolism

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Summary of the Problem

Newborn screening for inborn errors of metabolism (IEM) is a model of successful preventative medicine in public health.¹ Initiated over 48 years ago in the United States, it affords the opportunity to reduce mortality, morbidity and disabilities associated with treatable genetic metabolic conditions of the newborn. The technique of tandem mass spectrometry (MS/MS) now enables the diagnosis of over 30 genetic metabolic diseases.² In 2006, the American College of Medical Genetics called for the implementation of a uniform panel in order to establish a uniform standard of care for screening across the country.^{3,4} The primary treatment for about 30 such IEM depends on medical nutritional therapy involving the use of medical food. Therein lies one of the major weaknesses in the current provision of medical services for genetic metabolic conditions in the United States. Health plan coverage for screening is now well accepted but coverage for the subsequent treatment through the use of medical food is neither equitable nor uniform.⁵⁻⁹ This situation contributes to an inequitable delivery of healthcare services for diseases identified through newborn screening and confounds logic in that the development of successful dietary therapy for Phenylketonuria (PKU) provided the springboard for instituting universal newborn screening.¹⁰⁻¹³ It makes no sense to mandate newborn screening for metabolic disorders without ensuring that the follow-up, definitive diagnosis and particularly treatment are not included in the program design.

Background

What are inborn errors of metabolism

Over 700 (IEM) are known; they are hereditary defects that interfere with one or more biochemical functions that are essential for life. Disorders in the intermediary metabolism of protein, carbohydrate or lipids cause serious problems in infants and are most amenable to treatment with medical foods. In these disorders, either excess of one or more metabolites, or, conversely their lack from failure of endogenous synthesis, becomes critical and without correction of the metabolic abnormalities, severe systemic disease ensues. These disorders strike all sections of the population with an overall incidence of about 1:1500 but, individually, they are all rare. The best known is Phenylketonuria (PKU) that occurs in approximately 1:13,600 births. The national numbers are summarized in Table 1 which shows the numbers of patients born, those detected by NBS and an estimate of the total number of affected individuals in the US that require Medical Food therapy.

Essential role of medical foods for inborn errors of metabolism

After a child is diagnosed with an IEM that requires treatment with Medical Foods, the metabolic limitations and dietary requirements of the patients are first established based on the specific diagnosis and dietary requirements that support normal growth and development. Medical nutritional therapy is based on the principle that abnormal levels of metabolites in the blood cause serious or lethal complications that can be prevented by normalizing the blood biochemistry through manipulation of specific nutrients in the diet. The diet is crafted so that essential nutrients are provided from natural foods only in the amounts that will not create toxic accumulations of the offending metabolites. For a typical 10 year old child with PKU, this would require restriction to only 5 grams of natural protein which equates to about two slices of bread OR a half cup of milk. The rest of the diet MUST come from the medical foods to provide 1800 calories and 40 grams protein. Low protein medical foods contain negligible

amounts of protein in relation to their calorie content. This type of therapy intervention is a balancing act in which patient choice, cost, availability and insurance coverage all play an integral role. The more choice there is, the more likely that the child and family will adhere to the medical diet. Medical foods are therapeutic agents comparable to any regular drug and should be considered under the same rubric for prescribing and insurance purposes.

Types of medical foods

Medical foods come in three basic formats:

1. ***Infant formulas:*** For over 50 years the main products have used elemental forms of nutrients to make ersatz milk formula substitutes based on the composition of regular milk but lacking the “toxic” ingredients. Such products are ideal for use during infancy. However, a diet exclusively of milk is not feasible for normal adults and such products cannot provide a majority of the nutritional needs of older patients. An additional array of products for older children and adults is essential. These “milks” have been the main constituent in most metabolic diets and some people appear to consider them as the only legitimate form of medical food. This is clearly not the case.
2. ***Alternate protein products:*** As patients grow, infant formulas alone cannot to provide normal nutrition or any semblance of a normal existence. As a result novel formulations have emerged to provide the same essential protein and energy needs for older patients as the formulas do for infants. These include solid and powder forms of critical nutrients, amino acids in a variety of forms and protein free beverage powders that can be compounded into tailor-made diets that are somewhat more acceptable to older children, adolescents and adults. These products also exclude the specific nutrients that are harmful because of the primary diagnosis.
3. ***Low protein energy sources and alternate energy products:*** The above products are, by far, the most costly to manufacture. However, except for infants they are still do not provide adequate amounts of energy. They are designed as the main alternative to natural protein and thus are not suited to provide the total energy requirements. Adequate energy intake is just as important to a balanced diet as the control of the primary underlying metabolic defect. Inadequate energy intake can cause metabolic decompensation that can result in neurological damage just as severe as if treatment had never been started. Low protein substitute products come in the form of baking mixes, pastas, rice, sauces and pre-made items that are designed to be as similar to their normal counterparts as possible but yet supply negligible protein They become essential sources of energy, satiety and organoleptic satisfaction and markedly increase the chances of patient acceptance and compliance.

For other metabolic disorders, alternate energy sources are essential for preventing catabolism in order to avoid metabolic complications. An example is the inclusion of medium chain triglycerides in diet therapy which comes in the form of oil or powdered sachets with the addition of protein for the treatment of Fatty Acid Oxidation disorders.

Costs of medical foods

The combination of small volume manufacturing for a tiny market and often using unusual high cost ingredients costs more than normal high volume manufacturing. From infancy through 18 years of age, the annual costs of medical formula for PKU range from \$2,275 to \$12,48, averaging \$7,100 per year and \$220,000 from infancy through age 24 (Table 2). Depending on the pharmacy mark up the costs can be twice this amount in some areas of the country. There is also variation of costs among

the different disorders. The cost of treatment is higher for organic acidurias than PKU. See Table 3 for a cost comparison between PKU and Propionic Aciduria medical protein formulas and compared to regular infant formula. Other types of disorders cost less depending on the condition, the severity and treatment requirements. On average the low protein medical foods cost about 3-10 times more than their normal counterparts (Table 4). However, there are significant differences between the estimated costs of screening and treatment compared to costs of institutional care (Table 5).

The increased expenses of medical foods impose considerable strain on the budgets of most families confronted with the medical management of IEM. Health plan coverage provides the means to ensure that individuals access the necessary treatment modalities to prevent the complications of these genetic metabolic conditions.

In Appendix I, a list is provided of specific examples of the kinds of problems that patients and clinicians have been confronted with when dealing with denials of medical food coverage.

Why Healthcare Coverage for IEM is Impaired

There are several ways that the current system of healthcare imposes obstacles that interfere with the implementation of medical therapy for IEM that include the following:

1. Problems with coding for the diseases and their treatment
2. Variable State mandates and no federal guidelines regarding treatment
3. Effect of ERISA and TRICARE that trumps state mandates.
4. Insurance industry denial strategies

1) Problems with coding for the diseases and their treatment

For many of the IEM, there is no clear diagnostic ICD code and thus there is no evidence that the disorder even exists. The codes most frequently used to bill for medical foods are shown in Table 6. The HCPC codes create major problems for medical food reimbursement since none describes the realities of the situation. For most patients the metabolic formulas are consumed orally and are not administered by tube. Codes B4155, B4157 and B4162 all specify that the products be administered by tube. The B4157 code covers the use only of “nutritionally complete” products and thus excludes the nutritionally incomplete formulas specifically manufactured for IEM. B4155 comes closest to recognizing the nature of these medical foods but requires tube-feeding. B4162 does not indicate whether the products can be nutritionally complete or not. B4197 references parenteral nutrition, a term that is associated with IV therapy. Clearly the B codes do not recognize the current state-of-the-art either in regard to the diversity of products or in the ways that they can be used. It is true that a modifier, “BO”, is sometimes referenced with B4155, B4157, B4162 or B4197 codes designating that these treatments are taken orally thus “legitimizing” their use. However, this creates a gray zone that enables the opportunity for reimbursement denial depending on whether health plan providers are willing to overlook the inadequacy of the coding system. The restricted list of formulas associated with these B-codes does not allow for other formula powder options let alone different medical protein forms. Nowhere do the B codes reference the use of low-protein medical foods.

The S codes have been successfully used in the State of Oregon and elsewhere for reimbursement for both medical formulas and low protein foods. They were developed through collaboration between the Metabolic Clinic and Oregon BlueCross Blue Shield in 2001 after Oregon passed its first medical foods mandate in 1997. Other private and public health plans in the state then agreed to recognize these

codes. The Oregon Metabolic Clinic has set up a distribution system of medical foods for all conditions detected by newborn screening conditions that require medical nutritional therapy. Any appropriate foods that are labeled per FDA medical food guidelines for treatment of IEM can be included in the inventory. These products are distributed under medical supervision and billed to insurance using code S9435. This system has offered patients considerable freedom to individualize their treatment and has allowed daily variation in the diets as well as to reduce costs. In other states, Code S9434, that references modified solid foods has been used for low protein substitute products. In general however, S codes have limited utility in that they are viewed as temporary although they can be used indefinitely. They are typically recognized by private payers and in some instances by Medicaid but they are never processed for Medicare coverage and are not used in several states. In summary, the coding system lacks a mechanism that would allow appropriate coding for the modes of therapy that are currently in use.

2) *Variable state mandates and no federal guidelines regarding treatment*

Neither a federal mandate nor standard has been formally developed that encompasses a standard of care for metabolic diseases. However, 38 states have introduced legislation that mandate a wide selection of approaches; some requiring coverage of all products for all disorders, others only covering one disorder to some with restrictions that support caps and age limitations (Table 7) . Moving between states and changes of insurance coverage can be disastrous. The passage of SB1858 (Newborn Screening Saves Lives Act) lends urgency to the situation in that there is federal pressure to detect these disorders but no universal federal or state program to ensure proper treatment. Of the 38 states with mandates, 28 (74 %) include coverage of low protein foods in addition to the medical formulas. It should be noted that states with mandates have overwhelmingly supported coverage for formula and low protein foods by almost a 3:1 ratio (Figure 1). Since 2000, of 11/38 mandates that have been passed, all but two include coverage of low protein foods thus permitting far greater therapeutic flexibility and allowing for more cost savings.

Table 8 compares a selection of the specific metabolic disorders that are included in the core screening panel with the number of states that mandate coverage for medical food treatment for these conditions. It is certainly obvious that families confronted with the same disorder in different states, face significantly different financial realities. The states that have not passed mandates are listed in Table 9; funding supports services typically provide for treatment of PKU only through Medicaid or from other dedicated state funds. Four of the 12 stipulate treatment at least one other disorder than PKU. None of these states support treatment for all disorders identified through expanded Newborn Screening; although, 11 of these states have implemented expanded newborn screening for the 20 core metabolic disorders including Tyrosinemia. None of these states include low protein foods for coverage. This is an antithetical choice since increased reliance on the formulas with less emphasis on the low-protein leads to higher treatment costs (Table 10). It is less expensive to rely on a combination of medical protein sources in combination with low protein medical foods than to meet the majority of energy needs solely from a medical protein source. Ironically what may have started as a measure for cost savings, turns out in the long run to be the most costly approach to treatment.

State mandates

See Appendix II-IV for the texts of the Delaware, Oregon and Montana medical food legislative bills mandating medical food treatment.

3) *Effect of ERISA and TRICARE that trumps state mandates*

The Employee Retirement Income Security Act (*ERISA*) interferes with the States' ability to address shortcomings in healthcare coverage of medical foods by pre-empting legislative mandates. When ERISA was enacted through federal legislation passed in 1974 it essentially abolished State regulation of

employee benefit plans provided through self-insured employers. The purpose of ERISA was to protect enrollees of pension and benefit plans from abuse by those who invest and manage these plans. However, the law did not impose any requirements for healthcare benefits¹⁴ Standard health plans do not include benefits that expressly cover treatment requirements of rare conditions. Rare diseases are defined in the 1984 amendment to the Orphan Drug Act as - “any disease or condition which affects less than 200,000 persons in the US”.¹⁵ The 1989 report by National Commission on Orphan Disease highlighted the lack of adequate health insurance and coverage of medical expenses for these conditions.^{16, 17} Despite recognized standards for treatment of PKU with medical foods, denial of healthcare coverage is the norm for self-insured plans. It is the rare exception that a self-insured plan will respond positively to an appeal for medical food reimbursement. This creates a sense of injustice since the reality is that a with a different health plan program, medical food coverage for newborn screening disorders is not held to such rigid benefit policies. Thus ERISA upholds barriers that conflict with a state’s ability to address healthcare reform that supports coverage for standard of care treatment.

Inconsistent coverage by TRICARE and different Federal health plans create restrictions on portability and equal access to the recognized standard of care.

4) Insurance industry denial strategies

There are additional barriers to proper insurance coverage that include plain refusal by the companies to comply with state mandates and inability of parents to advocate for themselves effectively. Lack of enforcement for medical foods coverage by state insurance commissioners also contributes to a legal vacuum that ultimately erodes legislative intent.¹⁸ Even where adequate legislation exists, the health insurance industry regularly denies coverage creating an urgent need to engage in a demanding appeal process. Denials in healthcare coverage can result in the interruption to the supply of medical food or inconsistent use that can impact on control of metabolic disturbances associated with the disorder. The effects of this chaos are that families are called upon to argue medical necessity at a time when they have just had a newborn infant, when that infant is the most prone to biochemical damage and when they are coping with grieving and the medically management of a complicated disease. The appeal process can be daunting as it requires organizational skills, a sophisticated understanding of the disease in order to explain medical necessity and a clear understanding of the reimbursement process. Perseverance is the ultimate requirement. Clinical staff spends inordinate amounts of time in advocating on the behalf of patients with written testimony and telephone follow up that imposes a burdensome workload that erodes the time spent on actual patient care.

Federal Definition of Foods for Special Dietary Uses, Nutritional Supplements & Medical Foods

The FDA regulated “foods for special dietary uses” as drugs until 1972 under the Federal Food, Drug and Cosmetic Act.¹⁹ For purposes of promotion of product development, the FDA removed the drug classification and changed the categorization of medical foods to “foods for special dietary use”.²⁰ Further clarification as to what constitutes a medical food was established through Congressional action in the 1980s and FDA rules in the 1990s.¹⁹

The definition of what constitutes a Medical Food as opposed to a Nutritional Supplement is pivotal; the Federal definition of a medical food, is clearly laid out in the Orphan Drug Amendments of 1988 (section 5 (b)(3) of the Orphan Drug Act [21 USC 360ee (b)(3)]).

*“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."^{21, 22}

This definition clearly embraces almost all of the new products that are being marketed for treating IEM. It remains in sharp contrast to the Federal definition of a Nutritional Supplement which under the *Dietary Supplement Health and Education Act (DSHEA) of 1994* is as follows:

*"A dietary supplement is a product taken by mouth that contains a "dietary ingredient" **intended to supplement** the diet."*²³

The DSHEA specifically restricts labeling of nutritional supplements so claims cannot be made to suggest that the use of the dietary supplement will diagnose, prevent, mitigate, treat, or cure a specific disease (unless approved under the new drug provisions of the amended Food, Drug & Cosmetic Act, 1994).²⁴ In comparison, in May of 2007 the Center for Food Safety and Applied Nutrition, *Office of Nutritional Products, Labeling and Dietary Supplements* issued a guidance document for industry based on frequently asked questions about the definition of and regulations for medical foods.²⁵ In this document the explanation specifically detailed that medical foods --

1. *are distinguished from the broader category of foods for special dietary use and from foods that make health claims by the requirement that medical foods be intended to meet distinctive nutritional requirements of a disease or condition.*
2. *are used under medical supervision and intended for the specific dietary management of a disease or condition.*
3. *do not pertain to all foods fed to sick patients.*
4. *are foods that are specially formulated and processed (as opposed to a naturally occurring foodstuff used in a natural state) for the patient who is seriously ill or who requires the product as a major treatment modality.*
5. *must, at a minimum, meet the following criteria:*
 - a) *the product must be a food for oral or tube feeding;*
 - b) *the product must be labeled for the dietary management of a specific medical disorder, disease, or condition for which there are distinctive nutritional requirements;*
 - c) *the product must be intended to be used under medical supervision*²⁵

Dietary supplements augment the intake of particular nutritional components but cannot make the claim that the purpose is to treat a disease nor are they intended to sustain the user. In contrast, medical foods are the primary source of nutrition for patients with IEM and non medical foods are often the supplements. Medical foods and dietary supplements serve different purposes.

Other definitions in use include the following:

1. The USDA Center for Food Safety and Applied Nutrition [May 1997], *specifies that Medical Foods are prescribed by a physician, and cites as an example the treatment of PKU.*
2. The FDA Office of Nutritional Products states [May 2007] that Medical Foods are *"for patients with limited capacity to ingest, digest absorb or metabolize certain nutrients"*.
3. Orphan Medical Foods are defined as follows by the Orphan Drug Act [a] section 5 [b] [2] [3]: 1989: *"to treat a disease or condition that occurs so rarely that there is no reasonable expectation that a medical food for such disease will be developed without assistance"*.

Creating additional complexity, foods for **Special Dietary Uses** are defined by an international codex: *“for supplying particular dietary needs which exist by reason of a pathological or other condition including diseases, convalescence, pregnancy infancy or lactation”*---*“for supplementing or fortifying the usual diet with any vitamin, or other dietary property”* Code of Federal Regs: 21;2pt.105, April 1999

In the background information for The Medical Food and Food for Special Dietary Uses Act 2004 there is the following statement: *“It should be noted that the very same product may qualify as a Medical Food [e.g. in an institution] and at other times, if purchased at retail, does not qualify as a Medical Food”*.

Possible Solutions

1. Given the structure of the health care system in the US, it is essential to have a coding system that recognizes all forms of rare disease and allows for fair and appropriate reimbursement for the medical foods that are used for the treatment of IEM. A new system is essential.
2. It should be a given that if society implements a screening program the follow up and treatment should be factored into the program; universal expanded newborn screening in each state should guarantee equal commitment to treatment. An objective and rigorous review of the long-term efficacy of newborn screening in the US, would require an approach that includes collection of information on costs and benefits of short and long-term results that would include follow up and treatment.
3. State mandates that require treatment reimbursement for IEM should not be preempted by ERISA regulations. The fact that 74% of all mandates include both medical protein and low protein foods cannot be disregarded in the effort to build consensus on what is appropriate to be designated as medical food.
4. Federal standards for healthcare benefits should recognize the existence of rare diseases, contemporary approaches to their treatment and their relationships to the developmental needs of children. All types of medical foods should be included in a uniform panel of treatment for all disorders and at all ages.
5. Treating specialists should recognize that the Federal definition of medical foods is clear and includes all products that are manufactured expressly for treatment of IEM and are appropriately labeled as such.
6. Collaboration between the FDA, CMS and the insurance industry should explore mechanisms that would, once and for all, clarify the position of medical foods that are still regarded in a gray zone between straight pharmaceuticals and nutraceuticals. A definition that would specify the low protein options specifically intended for the treatment of inborn errors of metabolism would help to substantiate the premise that these options are essential foods and are not supplemental.
7. Pre-existing condition restrictions should be abandoned for Newborn Screening disorders so that continuity of care is not impeded.
8. Cost containment should not be an issue in establishing reimbursement standards that can, if the ceiling is too low, gut the intent of relieving the financial burden of treatment for these rare conditions.

| Table 1 – Population Estimates of IEM | |
|---|-------------|
| Births per year in the US | ~ 4 million |
| Rate of Detection of Inborn Error by expanded NBS | ~1:2,000 |
| Number requiring Medical Foods per year | ~1:1,500 |
| Current estimation of patients using medical foods in the United States | ~20,000 |

| Table 2. -- Average cost per year of medical protein options for PKU for different age groups | | | |
|--|--------------------------------------|------------------------|----------------------------|
| Age Group | Age Based Protein Requirement | Average Monthly | Yearly Average Cost |
| Infancy | 9.1-13.5 grams | \$190 | \$2275 |
| 1-3 years | 13 grams | \$273 | \$3275 |
| 4-8 years | 19 grams | \$429 | \$5150 |
| 9-13 years | 34 grams | \$718 | \$8617 |
| 14-18 years females | 46 grams | \$878 | \$10,538 |
| 14-18 years males | 52 grams | \$1040 | \$12,483 |

| Table 3 -- Retail cost of regular infant formula vs. wholesale cost of medical protein infant formulas.** | | | | | |
|--|-----------------------|------------------------------|--|------------------|----------------------------------|
| Regular Infant Formula | Regular Retail | Cost/100 grams Retail | Medical Protein Formula | Wholesale | Cost/ 100 grams wholesale |
| Similac Advance 365 grams, 12.8 oz. | \$14.99 | \$4.10 | Med. Protein – Infant Formula PKU 400 grams, 14 oz. | \$18.75 | \$4.69 |
| Enfamil Lipil, 366 grams; 12.8 oz | \$14.69 | \$4.01 | Med. Protein – Infant Formula-Propionic 400 grams, 14 oz | \$34.28 | \$8.57 |
| **Typical markup on medical food formulas is 200% to 300% of wholesale price sold through pharmacies | | | | | |

Table 4 -- protein content and cost comparison of regular foods vs. low protein alternatives per 100 gram weight of each type of product

| Regular Products | Retail Cost /100g | Grams of Protein/100 grams product | Low Protein Versions | Wholesale Costs/100 grams product | Grams of Protein/100 grams product |
|--------------------------|--------------------------|---|------------------------------------|--|---|
| | | 10-33x higher than low protein versions | | Wholesale 2 – 8x higher than retail for regular products | |
| Spaghetti | \$0.37 | 13 grams | Aproten Low Protein , Pasta | \$2.20 | 0.6 grams |
| Flour | \$0.17 | 10 grams | Wel-Plan Baking Mix | \$1.29 | 0.3 grams |
| Bisquick | \$0.31 | 7.5 grams | TC Low Protein Bake Mix | \$0.58 | 0.3 grams |
| Crackers | \$0.64 | 4 grams | Loprofin Crackers | \$1.95 | 0.4 grams |
| Tortillas, 8 pack | \$0.40 | 10 grams | Low- pro Tortillas, 6 pack | \$2.04 | 0.3 grams |
| Peanut Butter | \$0.70 | 25 grams | Low- pro Peanut Spread | \$1.94 | 0.4 grams |

| Table 5 – Estimated costs for detection, treatment vs. custodial care | |
|---|--------------------|
| Estimate of annual national cost of NBS @\$50/infant. State charges vary from \$25->\$100/infant | ~\$200, 000, 000 |
| Estimate of average costs for medical food treatment for 20,000 at ~\$5,000/person/year [This may be a high estimate] [for PKU it is \$7,100; for other disorders it can be less] | ~\$100,000,000 |
| Cost of Nursing Assisted Care in Residential Care Center/year/person * * http://oregoncares.org/legalfinancial/financial.html | \$50,000-\$100,000 |
| Estimate of cost of assisted care for 20,000 if untreated and cognitively disabled instead | Up to \$ 1 billion |

| Table 6 -- HCPC codes and description of products used to bill for medical foods | |
|---|---|
| HCPC Code | Description |
| B4155 | Enteral formula, nutritionally incomplete/modular nutrients includes specific nutrients, carbohydrates(e.g. glucose polymers),proteins/amino acids (e.g. arginine, glutamine), fat (e.g. medium chain triglycerides or combination; administered through an enteral feeding tube, 100 calories = 1 unit |
| B4157 | Enteral formula, nutritionally complete for special metabolic needs, for inherited disease of metabolism includes proteins, fats, carbohydrates, vitamins and minerals, may include fiber, administered through an enteral feeding tube. 100 calories = 1 unit |
| B4162 | Enteral formula for pediatrics, special metabolic needs for inherited disease of metabolism, includes proteins, fats, carbohydrates, vitamins and minerals, may include fiber, administered through an enteral feeding tube. 100 calories = 1 unit |
| B4197 | Parenteral nutrition solution; compounded amino acid and carbohydrates with electrolytes, trace elements and vitamins, including preparation, any strength, 74 to 100 grams of protein - premix |
| S9434 | Modified solid food supplements for inborn errors of metabolism |
| S9435 | Medical foods for inborn errors of metabolism |

Table 7—States with medical food mandates, year of passage, disorders specified for coverage, type of medical food covered, state or health insurance mandated coverage. ²⁶⁻³⁰

| | Year of Mandate | PKU Only | Mandated Coverage for Selection of IEM other than PKU | All IEM | Formula Only | Formula + Low Protein foods | State \$\$ Support | Private Payer Support \$\$ |
|----------------|------------------------------------|----------------------|---|----------------------------|---------------------------|--|--------------------------------|---|
| Alaska | 1991 | x | | | x | | | x |
| Arizona | 2000 | | X | | | x | | x |
| Arkansas | 1999 | | X | | | x | | x |
| California | 1999 | x | | | | x | | x |
| Colorado | 2001 | | | x | x | | | x |
| Connecticut | 1997 | | | x | | x | | x |
| Delaware | 2007 | | | x | | x | | x |
| Florida | 1995 | | | x | | x | | x |
| Hawaii | 1999 | | | x | | x | | x |
| Indiana | 2003 | | X | | x | | | x |
| Kansas | 1997 | | X | | x | | x | |
| Kentucky | 2002 | | | x | | x | | x |
| Louisiana | 2001 | | X | | | x | | x |
| Maine | 1995 | | | x | | x | | x |
| Maryland | 1995 | | | x | | x | | x |
| Massachusetts | 1993 | | X | | | x | | x |
| Minnesota | 1985 | | X | | | x | | x |
| Missouri | 2002 | | X | | | x | | x |
| Montana | 1999 | | | x | | x | | x |
| Nebraska | 1998 | | X | | | x | x | |
| Nevada | 1997 | | | x | | x | | x |
| New Hampshire | 1995 | | X | | | x | | x |
| New Jersey | 1997 | | | x | | x | | x |
| New Mexico | 2003 | | | x | | x | | x |
| New York | 1997 | | | x | | x | | x |
| North Carolina | 1997 | | X | | x | | x | |
| North Dakota | 2001 | | x | | | x | | x |
| Oregon | 1997 | | | x | | x | | x |
| Pennsylvania | 1996 | | x | | x | | | x |
| Rhode Island | 2008 | | x | | | x | | x |
| South Dakota | 1992 | x | | | x | | | x |
| Tennessee | 1996 | x | | | x | | | x |
| Texas | 1999 | | x | | x | | | x |
| Utah | 1998 | | x | | | x | | x |
| Vermont | 1998 | | | x | | x | | x |
| Virginia | 2000 | x | | | | x | x | |
| Washington | 1988 | x | | | x | | | x |
| Wisconsin | 1983 | | | x | | x | x | |
| Total | Out of a total of 38 states | 6 PKU Only | 16 Mandated Coverage for Some Disorders | 16 All Disorders | 10 Formula Only | 28 Formula + Low Protein foods | 5 State \$\$ Support | 33 Private Payer Support \$\$ |

Figure 1. Survey of States covering formula and foods with number of States covering PKU only, select disorders or all disorders compared to survey of States without mandates

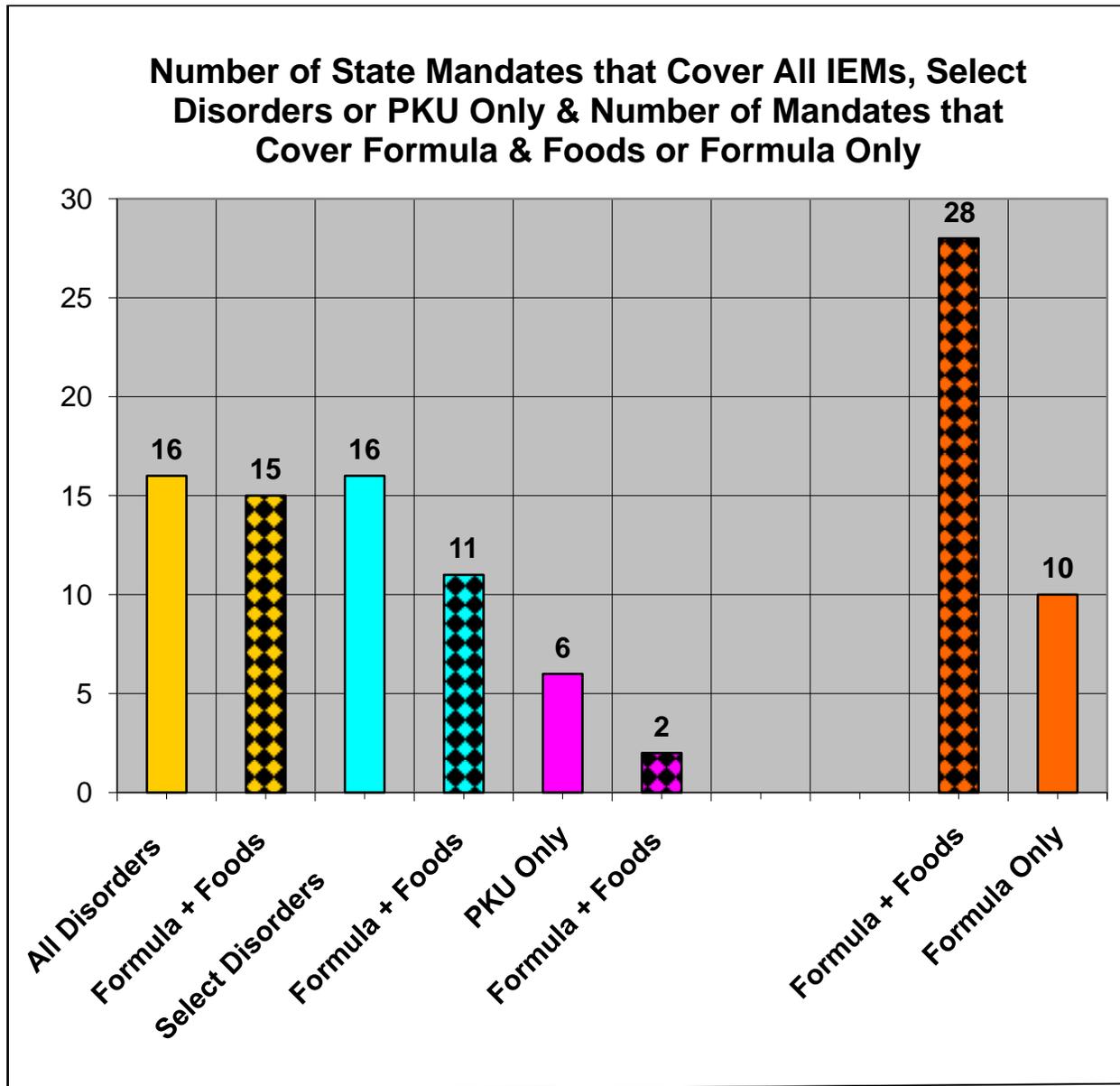


Table 8-- The number of states that screen for specific selection of genetic metabolic conditions as of May 18, 2009 and the number of states that mandate coverage for the treatment of these disorders

| Disease | Number of States that require full implementation of screening for disorder by law or rule | Number of States that Mandate Medical Food Coverage of same disorder |
|----------------|--|--|
| VLCAD & LCHAD | 50 | 17 |
| Homocystinuria | 50 | 18 |
| MSUD | 50 | 30 |
| Propionic | 50 | 27 |
| Citrullinemia | 50 | 26 |

| Table 9 -- States that have not passed mandates, list of funding sources that provide assistance for treatment, disorders covered and type of medical foods supported ²⁸⁻³⁰ | | | | | | | |
|---|-------------------|-----------------------|--|----------------------------------|---|---------------------|----------------------------|
| | No Mandate | State Coverage | | Medicaid Coverage | Other State Programs | Formula Only | + Low Protein foods |
| | | PKU only | Other Disorders | | | | |
| Alabama | X | x | no | Medicaid case by case | Children's Rehab Services | x | no |
| Georgia | x | x | no | Enteral not oral | Emory Univ. Children's Center | x | no |
| Idaho | x | x | no | Enteral not oral | Children's Special Health Program | x | no |
| Illinois | x | -- | yes | yes | May be considered upon by request State Formulary | x | no |
| Iowa | x | x | no | yes | Children's Special Health Care Services (CSHCS) | x | no |
| Michigan | x | x | no | Medicaid case by case | State Formulary; Children with Special Health Needs | x | no |
| Mississippi | x | x | no | Medicaid case by case | Children's Medical Program; State Formulary | x | no |
| Ohio | x | -- | HCU | yes | State Formulary; OH Department of Health Metabolic Program; Bureau for Children w/Medical Handicaps; Bureau of Early Intervention Program | x | no |
| Oklahoma | x | - | Individual consideration-case-by-case if not PKU | yes | Children w/Special Health Care Needs | x | no |
| South Carolina | x | x | no | Enteral not oral | State Formulary; Children's Rehab Services | x | no |
| West Virginia | x | - | Galactosemia | Referred to WV metabolic program | Department of Family Health- Metabolic Newborn Services (NBS) Program | x | no |
| Wyoming | x | x | no | yes | Genetic and Metabolic Clinic; SCHIP | x | no |
| Total | 12 | 8 | 4 | | | 12 | |

Table 10 --Treatment Cost Comparison for 9 year old diagnosed with PKU

Medical protein as primary energy source vs. combination therapy that includes medical protein and low protein medical foods

Nutrition requirements: 34 grams protein, 1950 calories;

Protein restriction: 6 grams, assume ~ 450 calories available from natural foods

| Medical Food Module | Medical Protein Per Day | Calories Supplied | Cost Per Day | Cost Per Month | Cost Per Year |
|--|--------------------------------|--------------------------|---------------------|-----------------------|----------------------|
| Med Bev XX 266 grams per day | 80 grams | 1500 | \$56 | \$1568 | \$18,816 |
| Medical Food Module | Medical Protein Per Day | Calories Supplied | Cost Per Day | Cost Per Month | Cost Per Year |
| Med Bev XX 113 grams/day | 34 grams | 463 | \$24 | \$672 | \$8064 |
| Low Protein Modules | ~2 grams | 1037 | \$8.60 | \$240 | \$2880 |
| Total | 36 grams | 1520 | \$32.60 | \$912 | \$10,944.00 |

Appendix I

Examples of problems in obtaining coverage for medical food treatment in different states

Colorado

Example 1): *We have a 25 yr old male with MSUD who struggled with compliance using conventional formula; however, he enjoyed the MSUD Coolers and become more compliant with intake. Unfortunately the code assigned to this product was based off of calories, so the reimbursement from Medicaid was too low for the DME to provide.*

Example 2): *We have a 13 yr old boy, who runs very high levels and has struggled with formula intake. He moved out of state and has recently returned. He has been taking the PhenylAde MTE AA blend for some time. Unfortunately when they returned to Colorado the reimbursement code has prevented his from continuing on the formula. This product is also based off of calories.*

Initially I thought the problem with our MSUD patient was that he was an adult as there are pediatric codes that are followed for most products. However, when I ran into the same problem with the 13 yr old I realized that the age didn't matter - just the code assigned to the product.

Currently the standard metabolic formulas in Colorado are assigned a code that reimburses a set percentage of the cost. However, when you move into the high protein/low calorie options the code changes to a specific reimbursement amount per 100 calories.

New York

New York State insurance law mandates coverage of both the medical food ('formula') and the low protein modified food products. The medical food is covered under the patients patient's pharmacy plan and most insurers (including Medicaid) comply with the exception of those employers whose health insurance benefits are self-funded, making them exempt (as a result of the Federal ERISA) from state insurance law.

The more frequent problem we find is getting the coverage for the low protein modified food. In Western New York, each of our major third party payers handles this issue differently. (As a case manager explained, 'The state mandates coverage but does not say how to do it!') Some patients pay out of pocket and submit the purchase order and proof of payment and then wait for reimbursement from the HMO. For others, the HMO accepts billing directly from the supplier. Each carrier limits the number of suppliers that the patients can order from.

Patients who have ONLY Medicaid cannot get coverage for the low protein foods. Most patients with Medicaid are encouraged to join an HMO and have their Medicaid administered that way: i.e., the HMO is their primary insurance with Medicaid secondary. To complicate matters even further, the HMOs 'carve out' the pharmacy portion, making Medicaid responsible. One of these HMOs is now denying coverage to patients with this type of Medicaid plans claiming that they are NOT responsible; yet the same HMO does cover the low protein foods under the medical portion of their plan for other patients who do not have Medicaid. Hence, to us it appears to be discrimination. People think that just because there is 'a law' that everything is going smoothly! Not so!

Each month, a new authorization is required for the medical beverage for a young child with PKU. The family must go through the authorization process anew and this process then interrupts care. Mom is extremely frustrated and has contemplated discontinuing treatment.

Appendix I – (continued)

Oregon/Washington

Aetna refused coverage for medical protein formula for an infant diagnosed with Propionic Aciduria. The family lives in Vancouver, Washington, whereas the metabolic clinic is in Oregon. The family was denied coverage on the basis that there wasn't a state mandate for medical formula and medical foods in Washington but only in Oregon and therefore there was no compelling reason to cover the medical food in this instance. (Propionic Aciduria is usually fatal if not treated). Washington's medical food law only mandates coverage for treatment of PKU, specifically only for medical protein formula. The funding problems for this family are ongoing due to a difference in job location and place of residence by a distance of 10 miles.

Oregon

An adult woman with PKU with cognitive delay exhibited rage and anxiety attacks in addition to severe eczema—all of which are associated with untreated PKU. She scratched her arms to the point that she was having problems with infections that then required antibiotic treatment, some of which she was allergic to. Her caretakers pursued dietary treatment with medical food but she was denied because her primary was Medicare which refuses coverage unless fed through a tube.

Texas

- **Private insurers using the lack of a specific diagnosis to deny benefits for medical food.**
 - ❖ *EXAMPLE: newborn with elevated blood tyrosine levels, responding to Tyrex-1 formula, pending biochemical/genetic confirmation. Until then, she has no official diagnosis and Blue Cross/Blue Shield of TX refuses to cover her medical food until she does.*
- **Private insurers flatly denying coverage for medical food for a child with a metabolic diagnosis, forcing the appeal process as a stall tactic.** *Blue Cross/Blue Shield of Texas is one of the worst offenders in this regard and TriCare is probably the second worst.*
 - ❖ *EXAMPLE: 11 month old with PKU diagnosed at birth in GA, was obtaining medical food without difficulty. Moved to TX and TriCare denied coverage for Phenex-1 & Phenex-2, requiring a letter of medical necessity from his metabolic geneticist for approval, a process which left them without treatment for several weeks.*
- **Pharmacies that refuse to order prescribed medical food products for patients.** *EXAMPLE: The same infant with PKU above—all 4 of TriCare's in-network pharmacies refused to order his medical food. A month later we are still waiting on TriCare to approve an out-of-network pharmacy with his medical food sitting on their shelves; in the interim we must call and beg Abbott for sample cans every week. Another child with a different defect had no difficulty getting the baby product (I-Valex-1) covered by Medicaid for her first 3 years. When we required transition to the next product, (I-Valex-2), the pharmacy stating Medicaid would only cover 1 of the 5 cans she requires each month (although with Medicaid, the same pharmacy had been 9 cans before this. In addition they demanded parents pay \$700/month for the other 4 cans (of infant formula). It took 3 weeks to help the family apply for WIC to get her I-Valex-2 (family had not been receiving WIC benefits previously) and transfer her I-Valex-1 prescription to a pharmacy that was willing to work with Medicaid to obtain Title IX coverage for I-Valex-2 above what WIC will provide monthly (and will stop providing when she turns 5).*

- **Exorbitant annual deductible and formula co-payment costs.** *Because these parents are working and have insurance, their income is too high for them to qualify for any form of government assistance and they don't have the insurance denial letters that all the private-sector patient assistance programs require.*
- **Process of coordination of benefits is confusing and inconsistent from patient to patient.** *Coordinating benefits between various state and federal programs including Medicaid, Medicare, WIC, CHIP and CSHCN (Title V), or between primary and secondary private insurance companies, takes up about 75% of our social worker's time and a good 15-20% of my time (for less than 100 patients) It seems the decision as to which of these programs covers what is different for each patient and varies depending on who you talk to; even if several patients have the same type of insurance from the same company, the process & end result are never the same. As our social worker and I attempt to iron things out case-by-case over several weeks to months, the patient and their family end up paying out of pocket for medical food with little or no assistance.*
- **Prolonged waiting periods for benefits.** *This is a particular problem for our patients who qualify for Title V (CSHCN) funds, as there is a one year waiting period before benefits are awarded*

Appendix II
The Delaware Medical Foods Bill

BE IT ENACTED BY THE GENERAL ASSEMBLY OF THE STATE OF DELAWARE:

Section 1. Amend Chapter 33, Subchapter III, Title 18, by inserting therein a new section, designated as §3355, which shall read as follows:

§3355. Phenylketonuria (PKU) and other inherited metabolic diseases.

(a) Definitions: In this section the following words shall have the meanings indicated:

(1) "Inherited metabolic diseases" shall mean diseases caused by an inherited abnormality of biochemistry. The words "inherited metabolic diseases" shall also include any diseases for which the State screens newborn babies.

(2) a. "Low protein modified formula or food product" means a formula or food product that is:

(i) specially formulated to have less than one (1) gram of protein per serving; and is

(ii) intended to be used under the direction of a physician for the dietary treatment of an inherited metabolic disease.

b. "Low protein modified food product" does not include a natural food that is naturally low in protein.

(3) "Medical formula or food" means a formula or food that is:

a. intended for the dietary treatment of an inherited metabolic disease for which nutritional requirements and restrictions have been established by medical research; and

b. formulated to be consumed or administered enterally under the direction of a physician.

(b) Application of this section. The provisions of this section shall apply to any health insurance contract that:

(1) provides coverage for a family member of the insured; and

(2) is delivered or issued for delivery in the State.

(c) A health insurance contract shall, under the family member coverage, include coverage for medical formulas and foods and low protein modified formulas and modified food products for the treatment of inherited metabolic diseases, if such medical formulas and foods or low protein modified formulas and food products are:

(1) prescribed as medically necessary for the therapeutic treatment of inherited metabolic diseases, and are:

(2) administered under the direction of a physician.

Section 2. Amend Chapter 35, Subchapter III, Title 18, by inserting therein a new section, designated as §3571, which shall read as follows:

§3571. Phenylketonuria (PKU) and other inherited metabolic diseases.

(a) Definitions: In this section the following words shall have the meanings indicated:

(1) "Inherited metabolic diseases" shall mean diseases caused by an inherited abnormality of biochemistry. The words "inherited metabolic diseases" shall also include any diseases for which the State screens newborn babies.

(2) a. "Low protein modified formula or food product" means a formula or food product that is:

(i) specially formulated to have less than one (1) gram of protein per serving; and is

(ii) intended to be used under the direction of a physician for the dietary treatment of an inherited metabolic disease.

b. "Low protein modified food product" does not include a natural food that is naturally low in protein.

(3) "Medical formula or food" means a formula or food that is:

a. intended for the dietary treatment of an inherited metabolic disease for which nutritional requirements and restrictions have been established by medical research; and

b. formulated to be consumed or administered enterally under the direction of a physician.

Appendix II page 2 The Delaware law

(b) Application of this section. The provisions of this section shall apply to any health insurance contract that:

- (1) provides coverage for a family member of the insured; and
- (2) is delivered or issued for delivery in the State.

(c) A health insurance contract shall, under the family member coverage, include coverage for medical formulas and foods and low protein modified formulas and modified food products for the treatment of inherited metabolic diseases, if such medical formulas and foods or low protein modified formulas and food products are:

- (1) prescribed as medically necessary for the therapeutic treatment of inherited metabolic diseases, and are:
- (2) administered under the direction of a physician.

Section 3. Amend Chapter 35, Subchapter IV, Title 18, by renumbering §§3571 – 3577 as §§3572 - 3578.

SYNOPSIS

Long Title: AN ACT TO AMEND TITLE 18 OF THE DELAWARE CODE RELATING TO HEALTH INSURANCE AND NEWBORN SCREENING.

Synopsis: This Bill provides that certain medical formulas and food expenses in the on-going treatment of Phenylketonuria (**PKU**) and other inherited metabolic diseases shall be covered in health insurance contracts and also in group and blanket health insurance policies.

PKU is one of a number of inherited metabolic diseases for which Delaware newborns are screened. **PKU** occurs in 1 in every 10,000 to 1 in every 25,000 live births. Other inherited metabolic diseases occur at similar or lower frequencies.

Undetected and untreated, **PKU** and other inherited metabolic diseases, can result in severe mental retardation, complicated medical conditions, extensive health care costs, or death. Such outcomes can be prevented by following a very strict, medically prescribed diet, which includes specially prescribed medical formulas or foods (such as low phenylalanine formula, and modified low protein foods). The preparation of these medical formulas and foods is complex and expensive.

Appendix III

The Oregon Medical Foods Bill

72nd OREGON LEGISLATIVE ASSEMBLY--2003 Regular Session

Senate Bill 74 Ordered by the House May 9

Including Senate Amendments dated March 25 and House Amendments dated May 9

Printed pursuant to Senate Interim Rule 213.28 by order of the President of the Senate in conformance with pre-session filing rules, indicating neither advocacy nor opposition on the part of the President (at the request of Joint Interim Committee on Judiciary for Senator Peter Courtney)

SUMMARY

The following summary is not prepared by the sponsors of the measure and is not a part of the body thereof subject to consideration by the Legislative Assembly. It is an editor's brief statement of the essential features of the measure.

{ - Exempts statute requiring - } { + Extends + } insurance coverage of certain metabolic disorders { - from automatic repeal provisions - } { + until July 3, 2009 + }. Declares emergency, effective July 3, 2003

A BILL FOR AN ACT

Relating to mandatory health insurance coverage for certain medical conditions; creating new provisions; amending ORS 743.726, 750.055 and 750.333; repealing ORS 743.726; and declaring an emergency.

Be It Enacted by the People of the State of Oregon: SECTION 1. ORS 743.726 is amended to read: 743.726. (1) All individual and group health insurance policies providing coverage for hospital, medical or surgical expenses, other than coverage limited to expenses from accidents or specific diseases, **shall include coverage for treatment of inborn errors of metabolism that involve amino acid, carbohydrate and fat metabolism and for which medically standard methods of diagnosis, treatment and monitoring exist, including quantification of metabolites in blood, urine or spinal fluid or enzyme or DNA confirmation in tissues. Coverage shall include expenses of diagnosing, monitoring and controlling the disorders by nutritional and medical assessment, including but not limited to clinical visits, biochemical analysis and medical foods used in the treatment of such disorders.** (2) As used in this section, 'medical foods' means foods that are formulated to be consumed or administered enterally under the supervision of a physician, as defined in ORS 677.010, that are specifically processed or formulated to be deficient in one or more of the nutrients present in typical nutritional counterparts, that are for the medical and nutritional management of patients with limited capacity to metabolize ordinary foodstuffs or certain nutrients contained therein or have other specific nutrient requirements as established by medical evaluation and that are essential to optimize growth, health and metabolic homeostasis. { + (3) This section is exempt from ORS 743.700. + }

Appendix IV

The Montana Medical Foods Bill

33-22-131. Coverage for treatment of inborn errors of metabolism. (1) Each group or individual medical expense disability policy, certificate of insurance, and membership contract that is delivered, issued for delivery, renewed, extended, or modified in this state must provide coverage for the treatment of inborn errors of metabolism that involve amino acid, carbohydrate, and fat metabolism and for which medically standard methods of diagnosis, treatment, and monitoring exist.

(2) Coverage must include expenses of diagnosing, monitoring, and controlling the disorders by nutritional and medical assessment, including but not limited to clinical services, biochemical analysis, medical supplies, prescription drugs, corrective lenses for conditions related to the inborn error of metabolism, nutritional management, and medical foods used in treatment to compensate for the metabolic abnormality and to maintain adequate nutritional status.

(3) For purposes of this section:

(a) "medical foods" means nutritional substances in any form that are:

(i) formulated to be consumed or administered enterally under supervision of a physician;

(ii) specifically processed or formulated to be distinct in one or more nutrients present in natural food;

(iii) intended for the medical and nutritional management of patients with limited capacity to metabolize ordinary foodstuffs or certain nutrients contained in ordinary foodstuffs or who have other specific nutrient requirements as established by medical evaluation; and

(iv) essential to optimize growth, health, and metabolic homeostasis;

(b) "treatment" means licensed professional medical services under the supervision of a physician.

(4) These services are subject to the terms of the applicable group or individual disability policy, certificate, or membership contract that establishes durational limits, dollar limits, deductibles, and copayment provisions as long as the terms are not less favorable than for physical illness generally.

(5) This section does not apply to disability income, hospital indemnity, medicare supplement, accident-only, vision, dental, or specified disease policies.

History: En. Sec. 1, Ch. 80, L. 1989; amd. Sec. 28, Ch. 451, L. 1993; amd. Sec. 56, Ch. 379, L. 1995; amd. Sec. 2, Ch. 434, L. 1999.

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