



COVERAGE OF MEDICAL FOODS FOR INHERITED METABOLIC DISORDERS (IMD) WILL SAVE LIVES & REDUCE COSTS

BACKGROUND

Inherited Metabolic Disorders (IMD), also referred to as Inborn Errors of Metabolism (IEM), are lifelong genetic conditions in which specific enzyme defects interfere with the normal metabolism of protein, carbohydrate, or fat. Thankfully, newborn screening programs identify the majority of IMD. However, coverage programs do not cover the cost of qualified medical foods and supplements, risking the health and financial security of families.

There are no cures for IMD, but medical nutrition allows patients to achieve normal or near-normal health.

Intervention treatment with medical food, foods modified to be low in protein, and/or supplementation with individual amino acids and large doses of vitamins must begin shortly after birth to prevent death, intellectual disability, and other adverse health outcomes. 22 of the 35 core conditions for which HHS recommends all newborns be screened must be treated with medical nutrition for life. **There is no alternative to medical nutrition treatment.**

Individually, IMD are rare. For example:

- Glutaric Acidemia Type 1 (GA-1) occurs in 1 in 92,300 live births
- Very Long-Chain Acyl-CoA Dehydrogenase (VLCAD) Deficiency 1 in 63,500 live births
- Phenylketonuria (PKU) 1 in 16,500 live births

However, the consequences of not treating these conditions are devastating. For example:

- **GA-1:** Metabolic crisis that can be fatal. Survivors can experience irreversible brain damage that can affect the ability to walk, talk or even swallow safely, often with uncontrolled, painful movements called dystonia.
- **VLCAD:** Metabolic crisis that can be fatal. Survivors can experience poor growth, liver failure, heart failure, and episodes of painful muscle breakdown that can cause kidney failure.
- **PKU:** Patients can experience irreversible cognitive impairment, hyperactivity, autistic behavior, seizures.
- **Maternal PKU Syndrome:** Affects children of women with poorly treated PKU and may include microcephaly (small brains), irreversible intellectual disabilities, congenital heart defects and other birth defects, and low birth weight.

The annual total cost to treat IMD with medical nutrition ranges from \$2,254 for an infant to almost \$25,000 for an adult or pregnant woman. Without coverage, treatment is unaffordable for the majority of patients. However, the cost of NOT providing accessible and appropriate treatment for these patients is much greater. Cost of care for an untreated PKU patient in an in-patient long-term facility approaches 105,850 U.S. dollars.¹

MEDICAL NUTRITION EQUITY ACT

Passage of the Medical Nutrition Equity Act (MNEA) will realize aims of the national newborn screening program: healthy lives for those born with devastating conditions. The MNEA would require health coverage of qualified, necessary medical foods. We call on Congress to address this issue by passing the bi-partisan MNEA.

Please contact your Members of Congress to co-sponsor the Medical Nutrition Equity Act today!

[1] B. L. Therrell, et al., Inborn errors of metabolism identified via newborn screening: Ten-year incidence data and costs of nutritional interventions for research agenda planning, *Mol. Genet. Metab.* (2014), <http://dx.doi.org/10.1016/j.ymgme.2014.07.009>

PEOPLE WITH PKU NEED MEDICAL NUTRITION TO THRIVE

Thousands of children and adults in the United States live with inherited metabolic disorders (IMD), also known as inborn errors of metabolism (IEM) that prevent their bodies from properly metabolizing and absorbing normal, everyday food. For these patients, medical nutrition is the primary treatment for the effective management of these conditions. Unfortunately, many health insurance plans in the United States do not provide reimbursement for medical nutrition despite their proven efficacy in the treatment of IMDs, causing medical nutrition therapy to be cost-prohibitive for many patients.

Necessity of Medical Nutrition for PKU:

- For more than 50 years in the United States, early medical nutrition intervention has resulted in near normal or normal development of individuals with PKU.
- Without access to medical nutrition, children with PKU can lose 4 IQ points each month and will suffer severe and irreversible intellectual disabilities before reaching toddlerhood.
- Adults who are not on treatment experience severe developmental, behavioral, and mental health consequences that result in difficulties in school, work, and relationships.
- Children carried by women with poorly-controlled PKU may have maternal PKU syndrome which causes small brains, intellectual disabilities, birth defects of the heart and low birth weight.

Cost of Medical Nutrition:

While medical nutrition is medically essential for PKU patients, it is not uniformly reimbursed by health insurance, creating a massive financial barrier in accessing treatment for many patients.

The out-of-pocket cost for medical nutrition products is up to 8 times the cost of normal groceries

	Standard	Low-Protein
Loaf of Bread	\$1.99	\$13.99
Box of Pasta	\$1.49	\$11.49
Box of Rice	\$2.49	\$11.00
Cake Mix	\$1.99	\$5.73
8 Bagels	\$2.29	\$13.49
32 Cheese Slices	\$3.39	\$13.49
2 Hot Pockets	\$2.00	\$9.99
Medical Formula (Primary source of nutrition)	N/A	\$300-\$1800/month

The Medical Nutrition Equity Act provides for the coverage of medical formula and low protein modified foods, as well as individual amino acids for children and adults with PKU and other metabolic disorders and certain digestive conditions under Federal health programs and private insurance.