HOW COVERAGE OF MEDICAL FOODS FOR INBORN ERRORS OF METABOLISM (IEM) CAN SAVE LIVES & COSTS

Inborn Errors of Metabolism (IEM), also referred to as Inherited Metabolic Disorders are lifelong genetic conditions in which specific enzyme defects interfere with the normal metabolism of protein, carbohydrate, or fat. Federal newborn screening policy and state screening programs identify the majority of IEM. Medical nutrition intervention is a mainstay of patient management and must begin shortly after birth to prevent death, intellectual disability, and other adverse health outcomes.

Individually, IEM 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

There are no cures for IEM, but treatment with medical food, foods modified to be low in protein, and/or supplementation with individual amino acids, and large doses of vitamins allow patients to achieve normal or near-normal health. **Twenty-two of the 34 core conditions on the HHS Recommended Uniform Screening Panel (RUSP) must be treated with medical nutrition for life. There is no alternative to this treatment.**

The consequences of not treating these conditions are devastating. For example:

- GA-1: Metabolic crisis that can be fatal. For survivors 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. For survivors poor growth, liver failure, heart failure, and episodes of painful muscle breakdown called rhabdomyolysis that can cause kidney failure
- PKU: 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
- Homocystinuria (HCY): Strokes that can be fatal. For survivors, paralysis, cognitive impairment, abnormal vision because of dislocated lenses, osteoporosis, and skeletal deformities

The annual total cost to treat IEM with medical nutrition ranges from $2,254 for an infant to almost $25,000 for an adult male 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.

The inequity of medical nutrition coverage across the United States has been, and continues to be, detrimental to individuals with IEM, their families, and society. In 2008, the Newborn Screening Saves Lives Act passed with overwhelming support. The Medical Nutrition Equity Act is the best way to achieve the aims of that act: healthy lives for those with IEM.

[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
[2] K.M. Camp, M.A. Lloyd-Puryear, K.L. Huntington, Nutritional Treatment for Inborn Errors of Metabolism: Indications, Regulations, and Availability of Medical Foods and Dietary Supplements Using Phenylketonuria as an Example, Mol Genet Metab. 2012 September; 107(1-2); 3-9. Doi:10.1016/j.ymgme.2012.07.005

Please contact the offices of Representative McGovern or Representative Herrera Beutler to co-sponsor HR 2501, or Senator Casey to become an original sponsor of the Senate bill.

PEOPLE WITH PKU NEED MEDICAL NUTRITION TO THRIVE

Thousands of children and adults in the United States live with 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 IEMs, 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, i.e.:
    - Metabolic Formula: ≥ $300.00 (per case of 6)
    - Low-protein modified foods:
        - Loaf of Bread: $13.99
        - Box of Pasta: $11.49
        - Cheese Slices: $13.40

The Medical Nutrition Equity Act of 2019 (H.R. 2501) 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 under Federal health programs and private insurance.

The Medical Nutrition Equity Act currently has 56 cosponsors with bipartisan support.

For more information, contact Kylie Barber at kbarber@curetheprocess.org

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