

January 31, 2023

Ms. Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244-1850

Re: CMS-9898-NC Request for Information; Essential Health Benefits

Submitted electronically at: <https://www.regulations.gov>

Dear Administrator Brooks-LaSure:

The undersigned patient and provider organizations appreciate the opportunity to respond to the Agency's request for information on Essential Health Benefits as published in the *Federal Register* on Dec. 2, 2022. Our organizations are united in efforts to improve coverage for and access to specialized formulas and medical foods —which we broadly classify as medically-necessary nutrition — for individuals with gastrointestinal (GI) and metabolic disorders. For patients with these disorders, medically-necessary nutrition is essential and, therefore, should be regarded as an essential health benefit (EHB).

MEDICALLY-NECESSARY NUTRITION IS ESSENTIAL

Certain GI and inherited metabolic disorders prevent the body from digesting, absorbing or metabolizing food. For these individuals, both children and adults, medically-necessary nutrition is a critical component of their medical treatment. The reasons for some of these conditions' reliance on medically-necessary nutrition is described below.

When these GI and metabolic conditions — that can be effectively managed with medically-necessary nutrition — are left untreated or under-treated, the implications are significant, often permanent and costly (monetarily and in quality of life). The effects are particularly profound in pediatric patients and include inadequate growth, abnormal development, cognitive impairment, and behavioral disorders. In severe cases, without medically-necessary nutrition, the outcomes may include unnecessary surgery, repeated hospitalizations, intellectual disability, or even death — all of which can be prevented with proper medical nutrition. Patients with an unmanaged disease also suffer emotionally and socially.

The 2022 formula shortage demonstrated the essential nature of specialty formulas for those with GI and metabolic conditions and the dire medical consequences that can result when these formulas are inaccessible. During the shortage, physician members of the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition (NASPGHAN) documented medical consequences such as feeding intolerance, weight loss, rectal bleeding, rapid gastric emptying, acute kidney injury, and electrolyte disturbances, all of which resulted in increased physician and emergency department visits. Children with metabolic disorders who were

hospitalized due to lack of formula faced similar challenges due to lack of formula supply and had to be placed on IV nutrition.

The irony of the federal government's urgent response to the formula shortage is that many health plans do not provide coverage for the same specialized formulas for patients with GI and metabolic disorders that were in shortage or difficult to access during the shortage period. Restoring supply does not improve access for those who can't afford their treatment.

Congress also recognized the essential nature of medical foods and formulas in the Consolidated Appropriations Act, 2023 by legislating steps to avert future shortages, which included defining all infant formulas and medically necessary foods as "critical foods" and providing new oversight and coordination through a newly established HHS "Office of Critical Foods." These steps will help avert a future shortage but barriers to access persist for many. The act recognized that while much of the language refers to "infant formulas," the definition of "critical foods" is wider and includes specialized formulas for inborn errors of metabolism and GI conditions that may be formulated specifically for affected adults.

The Biden-Harris Administration has also acknowledged the essential nature of formulas and other nutritional supports for children and adults with GI and metabolic disorders. We applaud their recognition as expressed in the recently-released "National Strategy on Hunger, Nutrition and Health," which states:

"The Administration also recognizes that for children and adults with digestive and inherited metabolic disorders, ensuring access to the right types of nutritional supports, such as infant formula, can prevent hospitalizations and be lifesaving. Still, there are often systematic barriers making access to these specialized nutritional supports challenging. CMS will evaluate coding, payment and administrative requirements in order to support access to nutritional supports for individuals with digestive and inherited metabolic disorders."

As CMS undertakes a review of the EHB, we ask that it take aim at the systemic barrier of access and affordability of medically-necessary nutrition for those with GI and metabolic disorders.

Treatment for Gastrointestinal Disorders

GI disorders for which specialized (non-standard) formulas may serve as an essential treatment component include: severe protein intolerance, allergies, malabsorption, and poor growth. For example, 2-5% of infants are affected by a milk protein allergy and require either hypoallergenic, extensively hydrolyzed or amino acid-based formulas as their sole source of nutrition — the same formulas that were in short supply in 2022.

Specialized formulas are also a primary modality for treatment of Crohn's Disease. Studies¹ in children have demonstrated that enteral therapy is as effective as conventional and biologic medication options in achieving remission in Crohn's Disease. There are few side effects

¹ Levine A, Wine E, Assa A. Crohn's Disease Exclusion Diet Plus Partial Enteral Nutrition Induces Sustained Remission in a Randomized Controlled Trial; *Gastroenterology*, <https://doi.org/10.1053/j.gastro.2019.04.02>

associated with enteral therapy, whereas pharmacologic treatments, including biological products, carry risk of medical complications, most often stemming from suppression of the immune system.

As another example, Eosinophilic Esophagitis (EoE) is an allergic disease in children and adults in which white blood cells build up in tissue, leading to inflammation, scarring, and narrowing of the esophagus. Symptoms may include poor weight gain (failure to thrive), difficulty/painful swallowing, vomiting after eating, and food refusal. Many individuals with EoE cannot tolerate a conventional diet; therefore, hypoallergenic or elemental formulas are the only opportunity for delivery of adequate nutrition. Studies have shown that when children are on exclusive enteral therapy, the inflammation of the esophagus can heal and the symptoms of EoE relieved without the need for pharmacologic therapy.

Oftentimes, when insurance companies do cover a medically necessary formula, it comes with the stipulation the formula be administered through a surgically placed tube at great cost to the health care system and increased risk of complications and infection to the patient who could otherwise consume the formula orally.

Treatment for Metabolic Disorders

The Federal Government's Recommended Universal Screening Panel (RUSP) has very specific requirements for a disorder's inclusion: that the impacts of the disorder be severe enough to warrant early intervention and that an effective treatment exists when a disorder is detected. For the majority of the Inherited Metabolic Disorders (IMD) on the RUSP, that recognized and accepted treatment is medically-necessary nutrition.

For the approximately 300 infants born each year with Phenylketonuria (PKU), medically-necessary nutrition is their primary—if not only—treatment. PKU is one of 13 aminoacidopathies, in which the body cannot metabolize one of the amino acids in protein, and patients must maintain a low-protein diet (often only 3-12 grams of natural protein) and consume a metabolic formula to provide the rest of their essential nutrition, for life. This treatment commences as early as possible after birth: failure to do so results in permanent and catastrophic brain damage. Lifelong treatment is necessary to avoid progressive neurological damage. In women with PKU who become pregnant, inadequate medically-necessary nutrition may lead to fetal brain damage, heart malformation, microcephaly and pre-term mortality.

In individuals with very long-chain acyl-CoA dehydrogenase deficiency (VLCAD) a metabolic crisis brought on by illness or lack of medically-necessary nutrition can be fatal. Survivors may experience poor growth, liver failure, heart failure, and episodes of painful muscle breakdown called rhabdomyolysis that can cause kidney failure.

MEDICALLY-NECESSARY NUTRITION AS AN EHB

The EHB could better address gaps in coverage for those with chronic and lifelong GI and metabolic conditions and disorders by ensuring coverage of medically-necessary nutrition, which includes medical foods and specialized formulas.

We ask for inclusion of medically-necessary nutrition as an EHB.

To comprehensively address the needs of patients with GI and metabolic disorders for which medically necessary food and formula constitute their treatment, we recommend the following definition of a medically necessary food:

Medically necessary food means food, including a low protein modified food product, an amino acid preparation product, a modified fat preparation product, or a nutritional formula that is a specially formulated and processed product (as opposed to a naturally occurring foodstuff) for the partial or exclusive feeding of an individual by means of oral intake or enteral feeding by tube; and intended for the dietary management of an individual who, because of a specified disease or condition, has limited or impaired capacity to ingest, digest, absorb, or metabolize ordinary foodstuffs or certain nutrients, or who has other special medically determined nutrient requirements, the dietary management of which cannot be achieved by the modification of the typical diet alone.

At a *minimum*, covered conditions should include:

- **Inherited metabolic disorders, including the following:**

Disorders classified as metabolic disorders on the Recommended Uniform Screening Panel Conditions list of the Secretary of Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children.

N-acetyl glutamate synthase deficiency

Ornithine transcarbamylase deficiency.

Carbamoyl phosphate synthetase deficiency.

- **Inherited disorders of mitochondrial functioning.**

- **Medical and surgical conditions of malabsorption, including the following:**

Impaired absorption of nutrients caused by disorders affecting the absorptive surface, functional length, and motility of the gastrointestinal tract, including short bowel syndrome and chronic intestinal pseudo-obstruction.

- **Malabsorption due to liver or pancreatic disease.**

- **Immunoglobulin E and non-Immunoglobulin E-mediated allergies to food proteins, including the following:**

Immunoglobulin E and non-Immunoglobulin E-mediated allergies to food proteins.

Food protein-induced enterocolitis syndrome.

Eosinophilic disorders, including eosinophilic esophagitis, eosinophilic gastroenteritis, eosinophilic colitis, and post-transplant eosinophilic disorders.

- **Inflammatory or immune mediated conditions of the alimentary tract, including the following:**

Inflammatory bowel disease, including Crohn’s disease, ulcerative colitis, and indeterminate colitis.

Gastroesophageal reflux disease that is nonresponsive to standard medical therapies.

ACCESS BARRIERS TO MEDICALLY-NECESSARY NUTRITION DUE TO COST

When insurance plans do not cover medically-necessary nutrition, the out-of-pocket costs to a patient or their family can be extraordinary. For individuals with certain GI and metabolic disorders, medically-necessary nutrition is as essential as insulin to manage diabetes or statins to lower cholesterol to prevent a heart attack or stroke. Medically-necessary nutrition is not a luxury for the individuals who rely on them — they are a validated medical treatment and are essential.

The Patients & Providers for Medical Nutrition Equity Coalition (PPMNE) has collected [patient and provider stories](#) that explain the tremendous cost burden associated with non-coverage of medically-necessary nutrition. The following are just a few examples:

Emeline

MARYLAND · PHENYLKETONURIA (PKU) · ANNUAL COSTS: \$5,400

“Having coverage for medical nutrition would mean so much to me and my family. As of now, my family pays about \$450 out-of-pocket for medical nutrition monthly, this adds up to about \$5,400 a year. All of this money going into my food has stopped us from being able to use it elsewhere. My sister is a rising senior in high school and since this money is going to my medical nutrition, we cannot put it towards her college experience.”

Remington

EOSINOPHILIC DISORDERS · FLORIDA · ANNUAL COSTS: \$25,200

“Remington was diagnosed with eosinophilic esophagitis as a toddler. ...the Walls family was fortunate to have insurance coverage for Remington’s formula through a self-funded insurance plan. When they had to change plans, the family was devastated to learn that coverage was no longer offered. The Walls family had no other option at that time than to pay out-of-pocket for the formula that Remington needed to survive. Remington is now approaching adulthood, and the amount of formula he needs to live has increased over the years, resulting in an estimated \$26,000 annual expense. That’s more than \$2,000 a month – the equivalent of an extra mortgage payment for some. In the fall of 2019, Remington headed off to college and is already taking into consideration how formula coverage could impact his hopes, dreams, and potential. In addition

to common stressors for teens his age, Remington also worries about how he will afford therapy and nourishment as a newly independent adult.”

Caroline, RD

“I am a Registered Dietitian for GI disorders. I spend probably 10-20% of every week working on ordering medical foods for patients with GI disorders like Crohn’s, Cystic Fibrosis, or Eosinophilic Esophagitis. Coverage across the Midwest is variable and this becomes very frustrating. As a provider, I know what is best for these patients and when covered, the results (i.e. improved absorption, reduced eosinophils, improved growth and weight gain) are nothing short of incredible. It is a waste of resources when these patients continue to be admitted to the hospital whereas if a simple formula change was made and the product was covered these patients could remain outpatient and healthy. I know of one pediatric patient who was seen at Mayo Clinic but who lived out of state whose family could not afford their medical formula for PKU and the patient was extremely ill as a result. This is a direct life or death example that this lack of coverage creates.”

GAPS IN COVERAGE OF MEDICALLY NECESSARY NUTRITION

Currently there is a patchwork of public and private payer coverage of medically-necessary nutrition. Where coverage exists, covered conditions are not uniform or payers impose unreasonable and medically-contraindicated stipulations, such as requiring a surgically placed feeding tube for the administration of formula that could be consumed orally.

As detailed above, coverage of medically-necessary nutrition is an example of a benefit that is essential to maintaining health, but insufficiently covered as an EHB.

State Laws and EHB

We appreciate the EHB-benchmark plan approach was designed to allow States to build on coverage that is widely available, minimize market disruption and provide consumers with familiar products. This approach does, however, create a patchwork of coverage of EHB, such that any benefit may have disparate coverage nationwide across all 51 EHB-benchmark plans. This is the case for medically-necessary nutrition for certain GI and metabolic disorders.

Upon review, at least eight EHB benchmark plans lack any coverage of medically-necessary nutrition for certain GI and metabolic disorders. Where coverage does exist, it is extremely variable. For example, some plans will only cover medically-necessary foods and formulas if they are the sole source of nutrition. Many children and adults with certain GI and metabolic disorders are able to supplement medical foods and specialized formulas on a limited basis with other sources of nutrition; yet, the medical food and specialized formulas constitute their treatment and a majority of their nutrition. This type of coverage restriction or those policies that only allow administration of enteral formula through a surgically placed tube create barriers to access. Also, where there is coverage, some benchmark plans cover medically necessary foods and formulas as a prescription drug benefit, while others classify coverage differently.

Examples of variability in current state and federal coverage:

Indiana

“Medical food that is Medically Necessary and prescribed by a Physician for the treatment of an inherited metabolic disease. Medical food means a formula that is intended for the dietary treatment of a disease or condition for which nutritional requirements are established by medical evaluation and formulated to be consumed or administered enterally under the direction of a Physician.”

New York

“Nutritional supplements (formulas) for the therapeutic treatment of phenylketonuria, branched-chain ketonuria, galactosemia and homocystinuria. Non-prescription enteral formulas for home use for which a Physician or other licensed Provider has issued a written order. The written order must state that the enteral formula is Medically Necessary and has been proven effective as a disease-specific treatment regimen for patients whose condition would cause them to become malnourished or suffer from disorders resulting in chronic disability, mental retardation, or death, if left untreated, including but not limited to: inherited diseases of amino acid or organic acid metabolism; Crohn’s disease; gastroesophageal reflux with failure to thrive; gastroesophageal motility such as chronic intestinal pseudo-obstruction; and multiple severe food allergies.”

WIC Coverage

Under WIC, coverage is available for those with qualifying conditions and there is no requirement that certain enteral products be the sole source of nutrition. WIC also allows for oral or enteral administration.

“WIC-eligible nutritional for participants with qualifying conditions (hereafter referred to as “WIC-eligible nutritional) means certain enteral products that are specifically formulated to provide nutritional support for individuals with a qualifying condition, when the use of conventional foods is precluded, restricted, or inadequate. Such WIC-eligible nutritional must serve the purpose of a food, meal or diet (may be nutritionally complete or incomplete) and provide a source of calories and one or more nutrients; be designed for enteral digestion via an oral or tube feeding; and may not be a conventional food, drug, flavoring, or enzyme. WIC-eligible nutritional include many, but not all, products that meet the definition of medical food in Section 5(b)(3) of the Orphan Drug Act (21 U.S.C. 360ee(b)(3)).”²

Under Food Package III, WIC-eligible nutritional are available to those with the following conditions:

“(i) Participant category served and qualifying conditions. This food package is reserved for issuance to women, infants and child participants who have a documented qualifying condition that requires the use of a WIC formula (infant formula, exempt infant formula or WIC-eligible nutritional) because the use of conventional foods is precluded, restricted, or inadequate to address their special nutritional needs. Medical

² 7 CFR 246.2 [https://www.ecfr.gov/current/title-7/subtitle-B/chapter-II/subchapter-A/part-246#p-246.2\(WIC-eligible%20nutritional%20for%20participants%20with%20qualifying%20conditions%20\(hereafter%20referred%20to%20as%20“WIC-eligible%20nutritional\)”\)](https://www.ecfr.gov/current/title-7/subtitle-B/chapter-II/subchapter-A/part-246#p-246.2(WIC-eligible%20nutritional%20for%20participants%20with%20qualifying%20conditions%20(hereafter%20referred%20to%20as%20“WIC-eligible%20nutritional)”))

documentation must meet the requirements described in [paragraph \(d\)](#) of this section. Participants who are eligible to receive this food package must have one or more qualifying conditions, as determined by a health care professional licensed to write medical prescriptions under State law. The qualifying conditions include but are not limited to premature birth, low birth weight, failure to thrive, inborn errors of metabolism and metabolic disorders, gastrointestinal disorders, malabsorption syndromes, immune system disorders, severe food allergies that require an elemental formula, and life threatening disorders, diseases and medical conditions that impair ingestion, digestion, absorption or the utilization of nutrients that could adversely affect the participant's nutrition status. This food package may not be issued solely for the purpose of enhancing nutrient intake or managing body weight.”³

Federal Employees Health Benefits Program (FEHBP)

Thanks to action by the Biden-Harris Administration, effective plan year 2023, all FEHB carriers will provide coverage for foods specifically formulated and prescribed to treat IEMs without regard to age, mode of administration (oral vs. nasogastric tube), narrow arbitrary limitations to specific diseases, or whether it is the sole source of nutrition for that individual. Our organizations support this expansion of medical foods for metabolic disorders and will be seeking further expansion of coverage to certain GI disorders.

TRICARE

In December 2016, Congress passed as part of the *National Defense Authorization Act of 2017*, legislative language that now ensures better and more consistent access to medically necessary foods for TRICARE beneficiaries with GI and metabolic disorders. Specifically, the NDAA requires TRICARE to cover medically necessary foods, and any supplies necessary to administer such food, and vitamins when prescribed for medical conditions of malabsorption; pathologies of the alimentary tract or the gastrointestinal tract; inborn errors of metabolism; and neurological or physiological conditions.

ACTUARIAL AND COST-SHARING LIMITATIONS

Actuarial considerations when evaluating the expansion of EHB to include medically-necessary nutrition for individuals with GI and metabolic disorders should include:

- Not all patients with GI disorders are treated with specialized formulas. In some cases, physicians will recommend pharmacologic treatment.
- Coverage of medically necessary nutrition creates treatment parity for GI disorders and metabolic disorders for which pharmacologic and biologic treatments are routinely covered, oftentimes at a greater cost and with an increased risk of adverse effects. For inherited metabolic disorders, payers routinely cover pharmacological treatments that cost 10 times the

³ 7 CFR 246.10(e)(3) [https://www.ecfr.gov/current/title-7/subtitle-B/chapter-II/subchapter-A/part-246#p-246.10\(e\)\(3\)](https://www.ecfr.gov/current/title-7/subtitle-B/chapter-II/subchapter-A/part-246#p-246.10(e)(3))

cost of medically-necessary nutrition but do not obviate the need for medical nutrition (most patients still require some medically-necessary nutrition).

- Coverage of medically-necessary nutrition reduces medical complications and poorer outcomes, and thus mitigates health care costs arising from those complications, for those with GI and metabolic disorders.
- Explicit, uniform and clear coverage requirements will reduce the administrative costs associated with the significant time medical providers devote to routinely appealing coverage decisions, many times unsuccessfully, to get approval of medically-necessary nutrition for their patients, often resulting in delayed access to treatment.

CONCLUSION

Our organizations are committed to ensuring equitable, fair and comprehensive coverage of medically-necessary nutrition to individuals with GI and metabolic disorders across payers and coverage policies. We are grateful for this opportunity to offer compelling stories and clinical rationale for why medically-necessary nutrition should be regarded as an EHB. Our community stands ready to serve as a resource to the Agency as it considers changes to EHB requirements. For questions or additional information, please contact Camille Bonta, NASPGHAN policy advisor, at (202) 320-3658 or cbonta@summithealthconsulting.com, or Sarah Chamberlin, Executive Director, National PKU News at sarah@pkunews.org or 973-619-9160.

Sincerely,

American Academy of Pediatrics
American College of Gastroenterology
American Gastroenterological Association
American Partnership for Eosinophilic Diseases
American Society for Parenteral and Enteral Nutrition (ASPEN)
Asthma and Allergy Foundation of America
Campaign Urging Research for Eosinophilic Diseases (CURED)
Children's National Hospital
Cincinnati Children's Hospital Medical Center
Color of Crohn's and Chronic Illness
Crohn's & Colitis Foundation
Dayton Children's
Digestive Disease National Coalition
Feeding Matters
Genetic Metabolic Dietitians International (GMDI)
Georgia PKU Connect
HCU Network America
International FPIES Association (I-FPIES)
MSUD Family Support Group
National Organization for Rare Disorders

National Pancreas Foundation
National PKU Alliance
National PKU News
Nationwide Children's Hospital
Network of Tyrosinemia Advocates
North American Society for Pediatric Gastroenterology, Hepatology and Nutrition
Organic Acidemia Association
Patients & Providers for Medical Nutrition Equity (PPMNE)
Propionic Acidemia Foundation
The FPIES Foundation
The New England Connection for PKU and Allied Disorders, Inc.
The Oley Foundation
United Mitochondrial Disease Foundation