**LETTER OF MEDICAL NECESSITY: PA**

**Date:**

**Patient:**

**D.O.B:**

**Policy Number:**

To Whom It May Concern:

This letter of medical necessity is regarding the nutrition management of **[PATIENT NAME].** This patienthas an inborn error of metabolism, a genetic disorder, known as **Propionic Acidemia (PA) (ICD 10: E71.121)** and is being followed in the genetic/metabolic clinic of **[CLINIC NAME].** This letter is to request coverage of **[CAMBROOKE PRODUCT NAME]** a medical food/formula(s) used for the treatment of PA.

PA results from a deficiency of the enzyme responsible for metabolizing the propiogenic amino acids; methionine, isoleucine, threonine, and valine. This deficiency results in the toxic build-up of organic acids in the body. Propionic acidemia is part of the newborn screening profile due to the devastating effects if undiagnosed or untreated. Untreated patients with PA have accumulations of organic acids leading to sever brain debilitation, multisystem organ failure and ultimately death. Normal growth and development are possible if an infant with PA is treated appropriately immediately upon diagnosis. However, these patients remain at high risk for developing episodes of metabolic crises that can be triggered by infection, injury, or failing to eat. Well controlled patients can still manifest growth, development, cardiac, ophthalmological, renal, and neurological complications.

The three primary goals of treatment are:

1. To maintain the blood levels of propiogenic amino acids to reduce toxicity to the brain, eyes, heart, and renal systems
2. To ensure that energy intake is sufficient and sustained throughout the day to prevent fasting and breaking down of body proteins
3. To ensure that the individual’s overall nutritional requirements are met, allowing for normal growth and development and the avoidance of nutritional deficiencies

There is currently no effective medication or gene therapy available for PA, however liver transplantation has been used to improved metabolic stability but is not a cure for PA. The standard of care for PA requires lifelong compliance with a diet restricted in propiogenic amino acids as well as odd-chain fatty acids and the consumption of special medical foods/formulas prescribed by a licensed physician. Some individuals with propionic acidemia may be supplemented with Biotin or Carnitine. This specialized medical nutrition management is medically supervised by a Geneticist and implemented by a registered dietitian specially trained in the nutrition management of inborn errors of metabolism. It requires frequent blood testing to control the blood levels of methionine, isoleucine, threonine, and valine, as well as close nutrition follow-up with the registered dietitian and medical team. The recommended treatment range of blood levels for individuals with PA is generally:

* Isoleucine: 25-105 μmol/L
* Methionine: 18-45 μmol/L
* Threonine: 45-250 μmol/L
* Valine: 65-250 μmol/L

For patients with PA there is a strong correlation between cognitive function and strict control of the propiogenic amino acids. Long term problems in a patient with PA that is chronically poorly managed can include poor growth, ataxia, cardiomyopathy, pancreatitis, learning problems, irreversible brain damage as well as severe and progressive neurological disorders. Currently, indefinite continuation of strict nutrition management is recommended for all patients with PA.

The specialized medical nutrition management for PA involves:

* A restricted diet that excludes all foods high in protein (e.g. meat, poultry, fish, eggs, cheese, dairy, nuts, and legumes) and markedly restricts all grains, including rice, breads, and pastas
* Special low-protein modified food products (which are not available in grocery stores)
* Sufficient calorie intake that is evenly distributed throughout the day to prevent catabolism
* Prescribed amino acid-based medical foods/formulas that are free of propiogenic amino acids to provide 55-60% of protein needs

The term medical food/formula, is defined in section 5(b) of the Orphan Drug Act {21 U.S.C. 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.”

Currently, this patient is prescribed **PROMACTIN AA PLUS,** which is a medical food/formula used to manage PA. The amino acids in medical food/formula are the primary protein constituent (in general 55-60% of protein needs) for the PA dietary treatment regimen. Medical nutrition therapy must also provide a sufficient and balanced intake of calories and other nutrients to avoid fasting and nutritional deficiencies. Nutrition therapy of PA without the use of medical foods is not possible, because it would cause severe protein malnutrition, calorie deprivation, vitamin and mineral deficiency, failure-to-thrive, and, ultimately, death.

**PROMACTIN AA PLUS** is only available by prescription through a pharmacy, durable medical equipment (DME) company, or directly from the manufacturer, Cambrooke Therapeutics, Inc. **[PATIENT NAME]** requires **[# OF GRAMS]** grams of protein equivalents per day from medical food/formula; therefore, we have prescribed **[# of tetras]** drink cartons of **PROMACTIN AA PLUS** per day (250 mL each) to meet the protein requirements for PA.

We appreciate your attention to this request for **PROMACTIN AA PLUS** medical food/formula(s), **[AMOUNT OF PRODUCT]** to be covered by their current medical insurance.

Please do not hesitate to contact us if you have any questions.

Sincerely,

**[Physician name, M.D.]**

**[Physician’s credentials, contact info, clinic name]**

**[Dietitian name, RD, LDN]**

Cc: **[Parents’ names]**

**References:**

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