

MEDICAL FOODS

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Medical foods are narrowly defined by the FDA under the Orphan Drug Act amendments as a food product specially formulated or processed to be used for the management of a disease or condition's specific nutritional needs or restrictions. The particular formulation and processing makes medical foods distinct from other foodstuffs consumed in their natural state. Medical foods are not simply those recommended by a physician for the management of a disease or condition. They are formulations developed specifically for the therapeutic or chronic management needs of a nutritional condition that could not be met through dietary modification alone, such as inborn errors of amino acid metabolism, food allergy, or an inability to meet nutritional needs orally.

Inborn errors of amino acid metabolism are, as implied by the name, metabolic disorders due to the inherited loss of an enzyme required for the metabolism of specific amino acids. For example, phenylketonuria (PKU) requires lifelong phenylalanine restriction and tyrosine supplementation in diet, while maple syrup urine disease requires only restriction of specific amino acids (leucine, valine, and isoleucine). As it would be nearly impossible to restrict specific components solely through naturally occurring foodstuffs alone, medical foods are often used to meet the nutritional needs of these patients.

Medical foods are also formulated to be hypoallergenic and can be used in the medical management of conditions where the specific allergen contributes to poor or worsening outcomes in the condition such as atopic dermatitis, gastrointestinal symptoms, IgE mediation, malabsorption disorder, seizure disorder, failure to thrive, or prematurity.

Specially formulated food may also be necessary to manage anatomic or structural issues, with or without a feeding tube, that inhibit food from reaching the small intestine, such as obstructing tumors, jaw fracture, or intestinal atresia. Functional inability to swallow foods or a significant risk of aspiration associated with chewing and swallowing food may also the use of medical food.

Summary

Medical foods are specially processed and formulated products intended for use under the supervision of a healthcare provider for the management or treatment of a condition that could not be managed through diet modification alone. Conditions such as food allergies, anatomical or

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functional variation that inhibits food from reaching the small intestine, or inborn errors of amino acid metabolism.

Prior approval is required to ensure the safe, clinically appropriate and cost-effective use of medical food products while maintaining optimal therapeutic outcomes.