



# HEALTHCARE NUTRITION COUNCIL

Improving outcomes through awareness and action

*Submitted via Regulations.gov*

April 8, 2022

Eleanor Dixon-Terry  
Office of Orphan Products Development  
Food and Drug Administration  
10903 New Hampshire Avenue  
Silver Spring, MD 20993-0002

RE: Healthcare Nutrition Council Comments on Docket Number FDA-2022-N-0116 Rare Disease Day Public Meeting

Dear Ms. Dixon-Terry,

The Healthcare Nutrition Council (HNC) is providing comments on Docket Number FDA-2022-N-0116 Virtual Public Meeting: FDA Rare Disease Day 2022. HNC is an association representing manufacturers<sup>1</sup> of enteral nutrition (EN) formulas and oral nutrition supplements (ONS), including those categorized as medical foods, and parenteral nutrition (PN). Our mission is to improve patient outcomes by advancing nutrition policies and actions that raise awareness and optimize access of essential nutrition support therapies across the continuum of care.

We appreciate the agency drawing much needed attention and awareness to rare diseases and the medical support needed for people with these conditions. Medical foods<sup>2</sup> play a critical role in supporting the nutritional needs of those with rare diseases. Inborn errors of metabolism are examples of metabolic conditions which prevent individuals from metabolizing nutrients in a normal way, necessitating the need for medical foods to meet the nutritional needs of the individual in order to prevent clinical complications and to sustain life. For example, phenylketonuria (PKU) is a condition, occurring in 1 in 10 – 15,000 infants in the U.S.,<sup>3</sup> which prohibits the individual from being able to metabolize phenylalanine causing it to accumulate in the body at harmful levels which could cause permanent cognitive impairment. Maple syrup urine disease (MSUD) is another inborn error of metabolism occurring in less than 1 in 185,000 infants worldwide.<sup>4</sup> In this condition, branched amino acids and the corresponding keto acids accumulate in the blood and can cause life-threatening seizures, coma, and brain damage.

In both of these conditions, it is impractical for individuals to modify their diets to carefully control their intake of the offending amino acid(s) while still meeting their nutritional needs, without the use of medical foods. Medical foods provide the nutrition needed to support growth and

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<sup>1</sup> HNC members are Abbott Nutrition, Nestle Healthcare Nutrition, and Nutricia North America.

<sup>2</sup> A medical food is "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." As defined in section 5(b)(3) of the Orphan Drug Act. 21 USC 360ee(b)(3).

<sup>3</sup> National Institutes of Health (NIH). U.S. National Library of Medicine. MedlinePlus. "Phenylketonuria." Retrieved from: <https://medlineplus.gov/genetics/condition/phenylketonuria/#frequency>.

<sup>4</sup> National Institutes of Health (NIH). U.S. National Library of Medicine. MedlinePlus. "Maple syrup urine disease." Retrieved from: <https://medlineplus.gov/genetics/condition/maple-syrup-urine-disease/#frequency>.

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development while managing the symptoms of their condition. PKU and MSUD are just two of many inborn errors of metabolism which require the use and consumption of medical foods.<sup>5</sup>

HNC published workshop proceedings<sup>6</sup> from our Medical Foods Workshop conducted in 2019 with the American Society for Nutrition. We ask FDA to consider the important role of medical foods in the management of certain rare diseases. HNC looks forward to future dialogue with the agency regarding the medical foods regulatory framework and opportunities for modernization to reflect the evolution of nutrition science, health care, and industry's ongoing innovations to formulate products specifically designed for the dietary management of diseases and conditions. We also hope to see improved access to medical food products, such as through health insurance coverage, so that people with these conditions can easily receive the life-sustaining nutrition they need.

Thank you for reviewing our comments. Please contact Berit Dockter MPP, RD, LD at [bdockter@healthcarenutrition.org](mailto:bdockter@healthcarenutrition.org) or 202-207-1112 if you have any questions.

Sincerely,

A handwritten signature in black ink that reads "Robert Rankin". The signature is written in a cursive, flowing style.

Robert Rankin  
Executive Director

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<sup>5</sup> Susan A. Berry, Christine S. Brown, Carol Greene, Kathryn M. Camp, Stephen McDonough, Joseph A. Bocchini and on behalf of the Follow-up and Treatment (FUTR) Workgroup for the Advisory Committee on Heritable Disorders in Newborns and Children. Medical Foods for Inborn Errors of Metabolism: History, Current Status, and Critical Need. *Pediatrics*. March 2020, 145 (3) e20192261; DOI: <https://doi.org/10.1542/peds.2019-2261>.

<sup>6</sup> Holmes, Jennifer L.; Biella, Alexandre; Morck, Timothy; Rostorfer, Jena; and Schneeman, Barbara. (2021). Medical Foods: Science, Regulation, and Practical Aspects. Summary of a Workshop. *Current Developments in Nutrition*, Volume 5, Issue Supplement 1. <https://doi.org/10.1093/cdn/nzaa172>.