Abetalipoproteinemia and Related Disorders Foundation (ABL+ Foundation)

Template Letter of Medical Necessity for VITAMIN A Coverage

Patients with abetalipoproteinemia and other familial hypobetalipoproteinemia disorders possess gene mutations required to package lipids into particles for absorption and transportation throughout the body (1, 2). Fat-soluble vitamins, including vitamins E, A and K are also dependent on these pathways for absorption. Consequently, patients develop severe deficiencies of these vitamins and the clinical outcomes can be catastrophic, including debilitating neurologic impairment, blindness and life-threatening bleeding.

Vitamin A deficiency in patients with abetalipoproteinemia and other hypobetalipoproteinemia disorders can result in retinal degeneration, night blindness and progressive vision loss. If left untreated, patients may develop legal blindness(3). An exceptionally high dose of vitamin A (100-400 IU/kg/day) is medically necessary for patients with abetalipoproteinemia and other familial hypobetalipoproteinemia disorders (2).

For patients with abetalipoproteinemia and other hypobetalipoproteinemia disorders, vitamin A is not a micronutrient supplement, instead an essential therapeutic medication.

As the ABLRDF Medical Advisory Panel, our members include established researchers and medical professionals who care for patients with these disorders. We attest that a high dose of vitamin A taken lifelong is medically necessary to prevent devastating complications in patients with abetalipoproteinemia and other hypobetalipoproteinemia disorders.

Sincerely,

Dins Slark

Dennis D. Black, MD, AGAF J. D. Buckman Professor of Pediatrics Professor of Physiology Vice Chair for Research University of Tennessee Health Science Center Director, Children's Foundation Research Institute Vice President for Research, Le Bonheur Children's Hospital Room 471R, Children's Foundation Research Tower 50 North Dunlap Memphis, TN 38103 Phone: 901-287-5355 FAX: 901-287-4478 E-mail: dblack@uthsc.edu

20

Cindy Bredefeld, DO, FACE

Attending Physician, Division of Endocrinology, Diabetes & Metabolism Diplomate, American Board of Clinical Lipidology Director, Diabetes Clinical Trials Assistant Professor of Clinical Medicine, NYU Long Island School of Medicine

cindy.bredefeld@nyulangone.org

N Bri

Mitchell F. Brin, MD, FAAN, FANA, FAHS Clinical Professor of Neurology University of California, Irvine mbrin@uci.edu

Richard J. Deckelbaum, MD, CM, FRCP(C)

Robert R. Williams Professor of Nutrition Professor of Pediatrics, Professor of Epidemiology Director, Institute of Human Nutrition Vagelos College of Physicians and Surgeons Columbia University Irving Medical Center New York, NY

M. Mahmood Hussain President Abetalipoproteinemia & Related Disorders Foundation Professor and Endowed Chair Director, Diabetes and Obesity Research Center Department of Foundations of Medicine NYU Long Island School of Medicine NYU Langone Hospitals – Long Island

101 Mineola Blvd., Mineola NY

1. Bredefeld C, Peretti N, Hussain MM, and Medical Advisory P. New Classification and Management of Abetalipoproteinemia and Related Disorders. *Gastroenterology*. 2021;160(6):1912-6.

- 2. Bredefeld C, Hussain MM, Averna M, Black DD, Brin MF, Burnett JR, et al. Guidance for the diagnosis and treatment of hypolipidemia disorders. *J Clin Lipidol*. 2022.
- 3. Segal S, and Sharma S. Ophthaproblem. Vitamin A and vitamin E. *Can Fam Physician*. 2005;51:1079, 85-6.