

Updated May 3, 2022

## AGENDA

All times are Eastern (EDT UTC-4)

9:30 – 9:40

### Welcome

**Brenda Stodart, PharmD, BCGP, RAC-US**

Captain, United States Public Health Service  
*Director, Small Business, and Industry Assistance (SBIA)*  
Division of Drug Information (DDI) | Office of Communications (OCOMM) | CDER

9:40 – 9:55

### FDA Introduction

- Multifactorial pathophysiology of PNALD/IFALD
- Risk associated with phytosterols
- Regulatory History

**Judy Racoosin, MD, MPH**

*Deputy Director for Safety*  
Division of Hepatology and Nutrition  
(DHN)  
CDER

## Session 1: Scope of the Problem

Moderator: **Gerri Baer, MD** Supervisory Medical Officer, Office of Pediatric Therapeutics, Office of the Commissioner

9:55 – 10:15

### Neonates and Infants

- Current utilization patterns of intravenous lipid emulsion (ILE) treatment in neonates
- Changing incidence and epidemiology/natural history of PNALD/IFALD over the past decade in the neonatal population;
- Strategies to manage PNALD/IFALD

**Camilia R. Martin, MD MS**

*Associate Professor of Pediatrics*  
*Chief, Division of Newborn Medicine*  
New York-Presbyterian/Weill Cornell  
Medical Center

10:15 – 10:35

### Older Pediatric Patients

- Current utilization patterns of ILE treatment in older children
- Changing incidence and epidemiology/natural history of PNALD/IFALD over the past decade in older children
- Strategies to manage PNALD/IFALD

**Samuel A. Kocoshis, MD**

*Professor, University of Cincinnati Department of Pediatrics*  
*Medical Director, Small Bowel Transplantation Program*  
*Medical Director, Intestinal Care Center*

10:35 – 10:55

### Adults

- Current utilization patterns of ILE treatment in adults
- Changing incidence and epidemiology/natural history of PNALD/IFALD over the past decade in adults
- Strategies to manage PNALD/IFALD

**Alan Buchman, MD, MSPH**  
*Professor of Clinical Surgery and Medical Director*  
Intestinal Rehabilitation and Transplant Center  
University of Illinois at Chicago

10:55 – 11:05: BREAK

11:05 – 12:00

### Panel Discussion

- How big a problem is PNALD/IFALD in 2022?
- Are specific populations at higher/lower risk?

**Camilia R. Martin, MD**  
**Samuel A. Kocoshis, MD**  
**Alan Buchman, MD, MSPH**

**David F. Mercer MD, PhD**  
*Professor of Surgery*  
*Director of Intestinal Rehabilitation Program*  
University of Nebraska Medical Center

**M. Petrea Cober, PharmD**  
*Professor, Pharmacy Practice*  
*Director of Professional Development*  
Northeast Ohio Medical University  
(NEOMED)  
College of Pharmacy

12:00 – 12:30: LUNCH BREAK

## Session 2: Role of phytosterols as a modifiable risk factor in the pathophysiology of PNALD/IFALD; where can we intervene?

Moderator: **Ruby Mehta, MD** Acting Team Leader, DHN

12:30 – 1:00

### Metabolism of Phytosterols Non-clinical (Animal) Evidence Supporting the Association Between Plant-based ILEs and PNALD/IFALD

**Douglas Burrin, Ph.D.**  
*Research Physiologist*  
United States Department of  
Agriculture  
Agricultural Research Service  
Children's Nutrition Research Center  
Department Pediatrics, Baylor College  
of Medicine

1:00 – 1:20

### Clinical Evidence Supporting the Association Between Plant-based ILEs and PNALD/IFALD

**Kishore R Iyer, MBBS**

*Director, Intestinal Rehab & Transplant Program  
Program Director, Transplant Surgery  
Fellowship  
Professor of Surgery & Pediatrics  
Mount Sinai Medical Center  
Icahn School of Medicine at Mount Sinai*

1:20 – 1:40

### Scientific Challenges to Producing an ILE with Reduced Phytosterol Content

**David F. Driscoll, Ph.D.**

*Vice President of Stable Solutions, LLC  
Associate Professor of Medicine,  
UMASS Medical School*

1:40 – 1:55

### Industry Perspective - Fresenius Kabi

**Martin Westphal, MD, MBA**

*Chief Medical Officer and Executive VP  
Medical, Clinical & Regulatory Affairs, BU Nutrition  
Fresenius Kabi*

1:55 – 2:10

### Industry Perspective - Baxter International Inc.

**Baptiste Bodet, M. Sc. Chemical Engineering**

*Baxter International Inc.  
Technical Leader | Clinical Nutrition BU*

**2:10 – 2:20: BREAK**

2:20 – 4:20

### Panel Questions and Discussion

- Does the non-clinical and clinical evidence support phytosterols as a dose related hepatotoxin?
- Is a phytosterol-depleted lipid formulation scientifically feasible?
- Is demonstrating a low phytosterol content adequate to establish safety of an ILE or must such a product be demonstrated to have a safety advantage in a clinical trial?
- Clinical trial issues of concern
- Are there other modifiable risk factors for PNALD/IFALD besides phytosterol exposure?
- What interventions would you recommend to minimize the risk of PNALD/IFALD?

**Douglas Burrin, PhD**

**Kishore R Iyer, MBBS**

**David Driscoll, PhD**

**Valeria C. Cohran, MD, MS**

*Medical Director, Intestinal Rehabilitation and Transplant  
Associate Professor of Pediatrics  
Feinberg School of Medicine  
Northwestern University*

**Muralidhar H. Premkumar, MBBS**

*Associate Professor, Pediatrics and Neonatology  
Baylor College of Medicine*

**4:20 – 4:30: WRAP-UP**