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