



# Medical Foods: Opportunities in an emerging market

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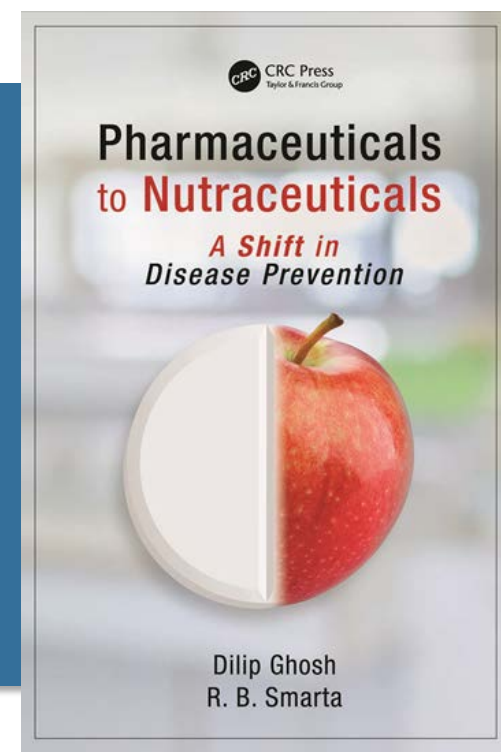
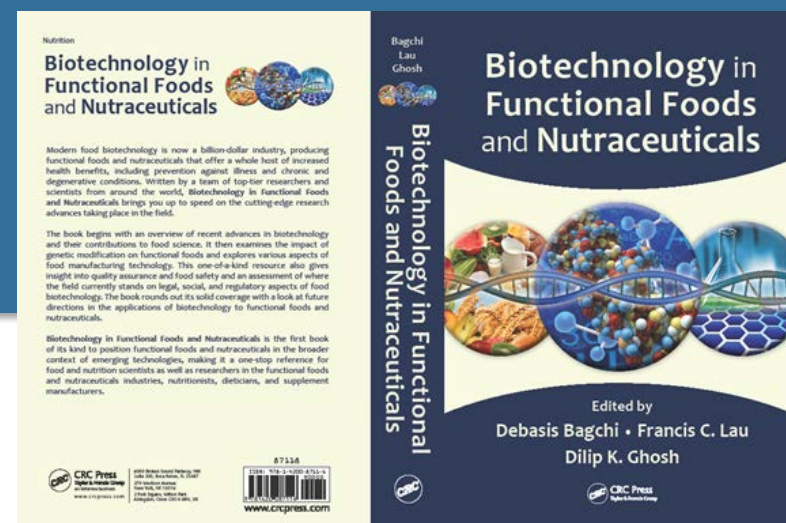
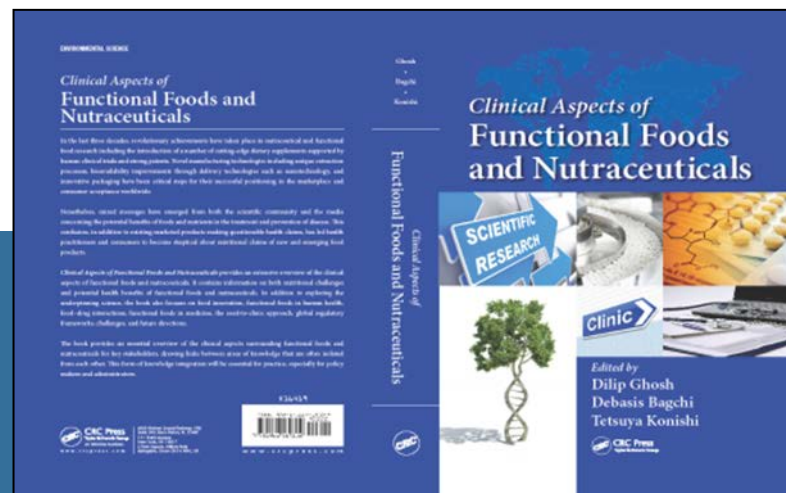
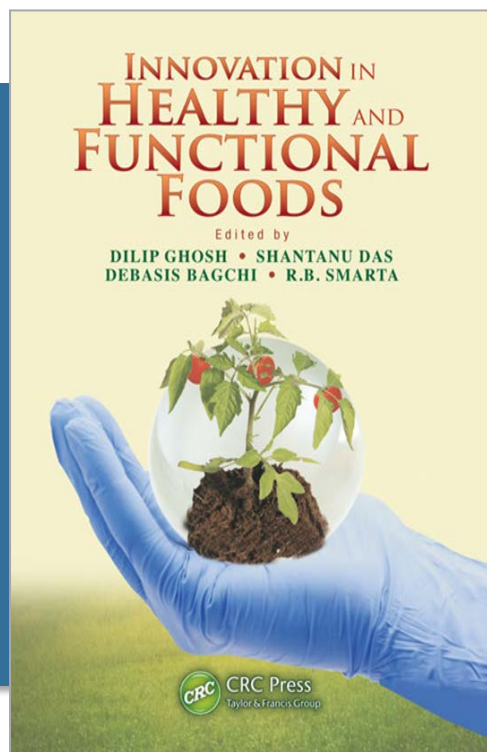


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NO  
IMAGE

# The Future of Dietary Supplements and Personalized Nutrition

[Dilip Ghosh](#)

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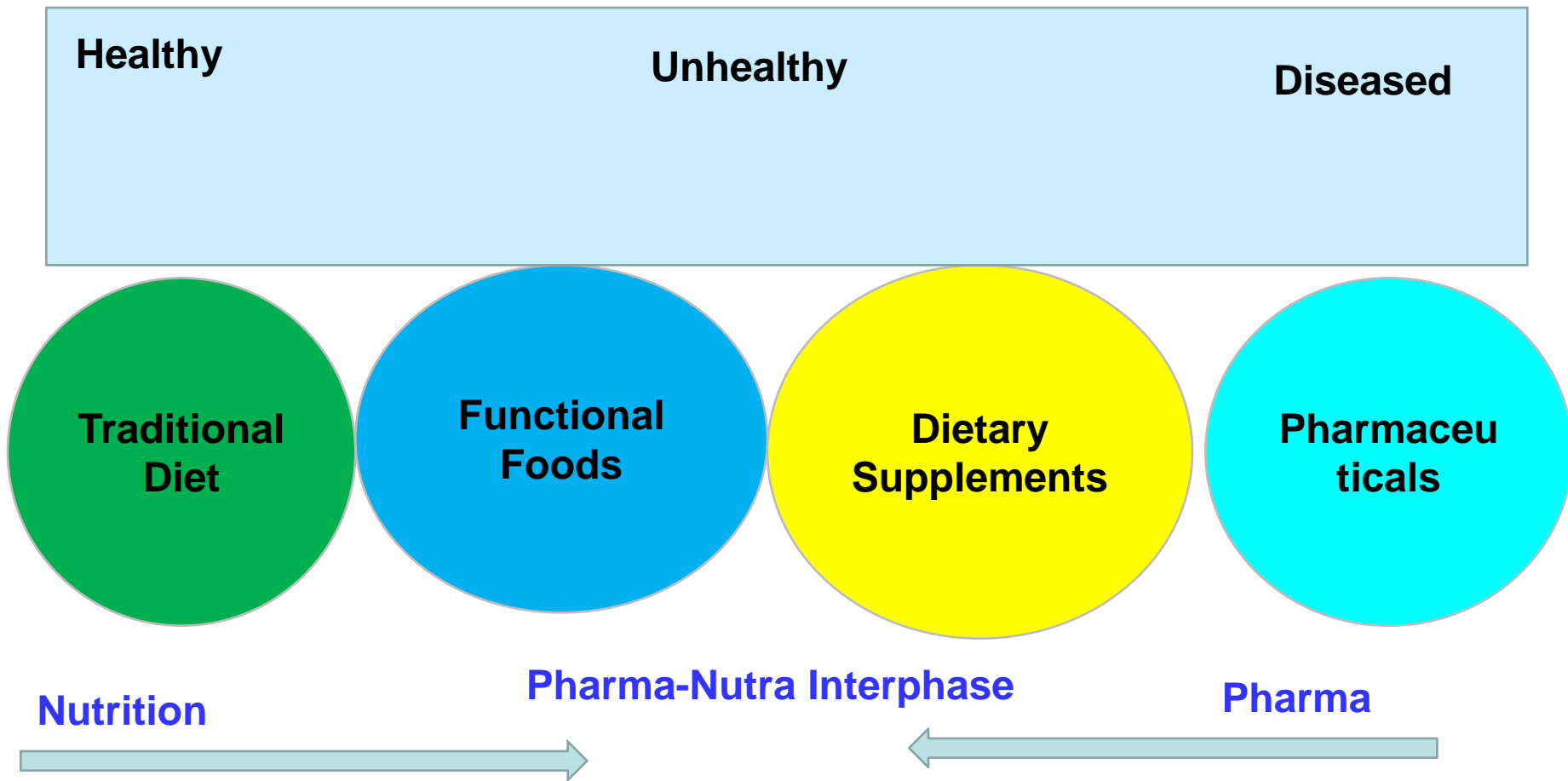
352 pages

July 2017, Wiley-Blackwell

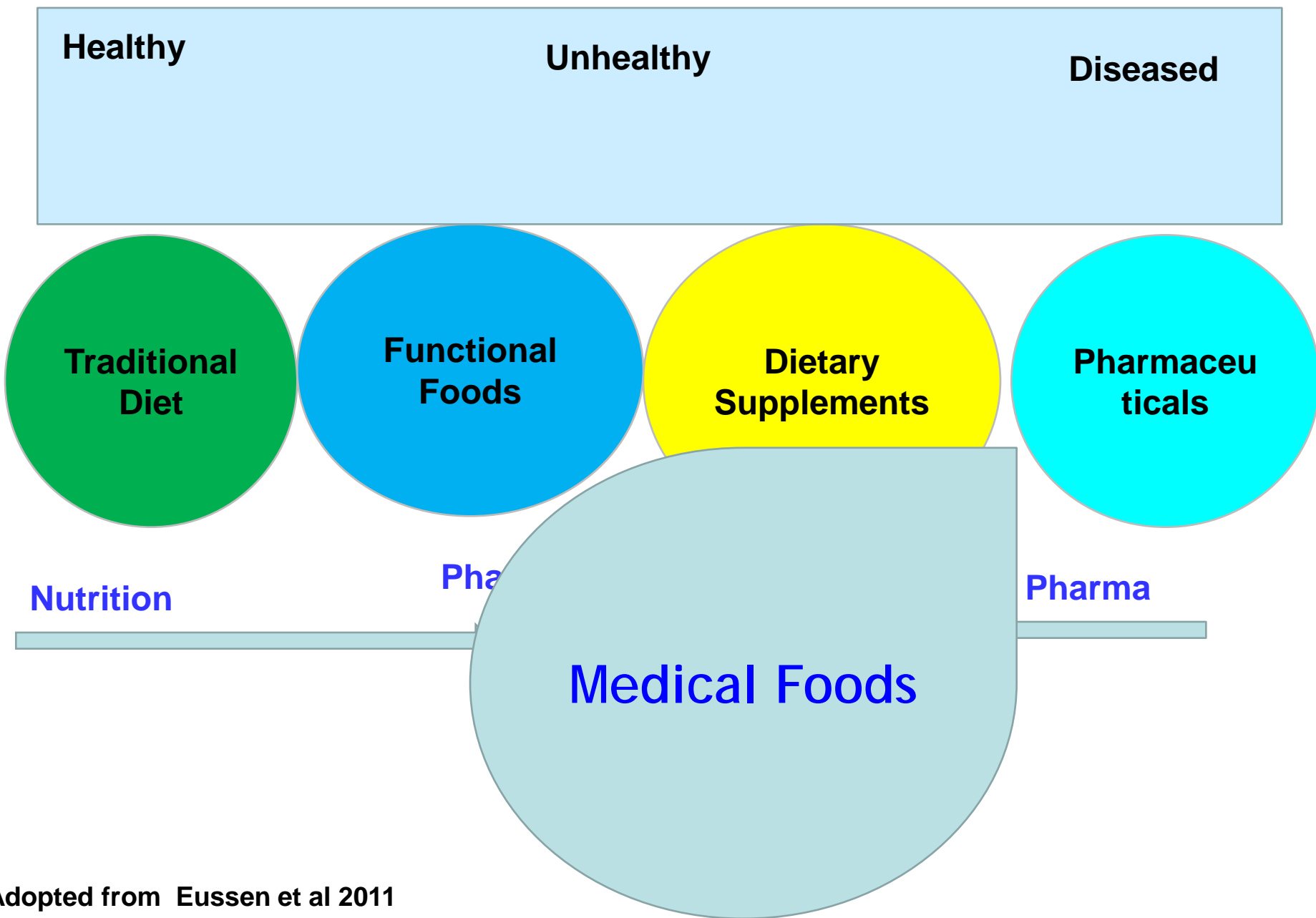
# Medical Foods - Overview

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- **A unique category of FDA regulated products that was established in 1983 and is defined in section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3))**
- **Medical Food Requirements:**
  - Must be used under the supervision of a physician
  - Must meet the distinctive nutritional requirements or metabolic deficiencies of a particular disease that cannot be met with a simple dietary shift
  - Must be safe– all ingredients in a Medical Food must be Generally Recognized As Safe (GRAS)
  - Must be manufactured according to Current Good Manufacturing Practices (cGMP) regulations



Adopted from Eussen et al 2011



Adopted from Eussen et al 2011

# Medical Foods

like Odysseus sailing between Scylla and Charybdis

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Medical Foods (Odysseus)

Prescription drugs (Scylla)



Nutraceuticals and dietary  
supplements (Charybdis)



# Medical Foods - A Comparison

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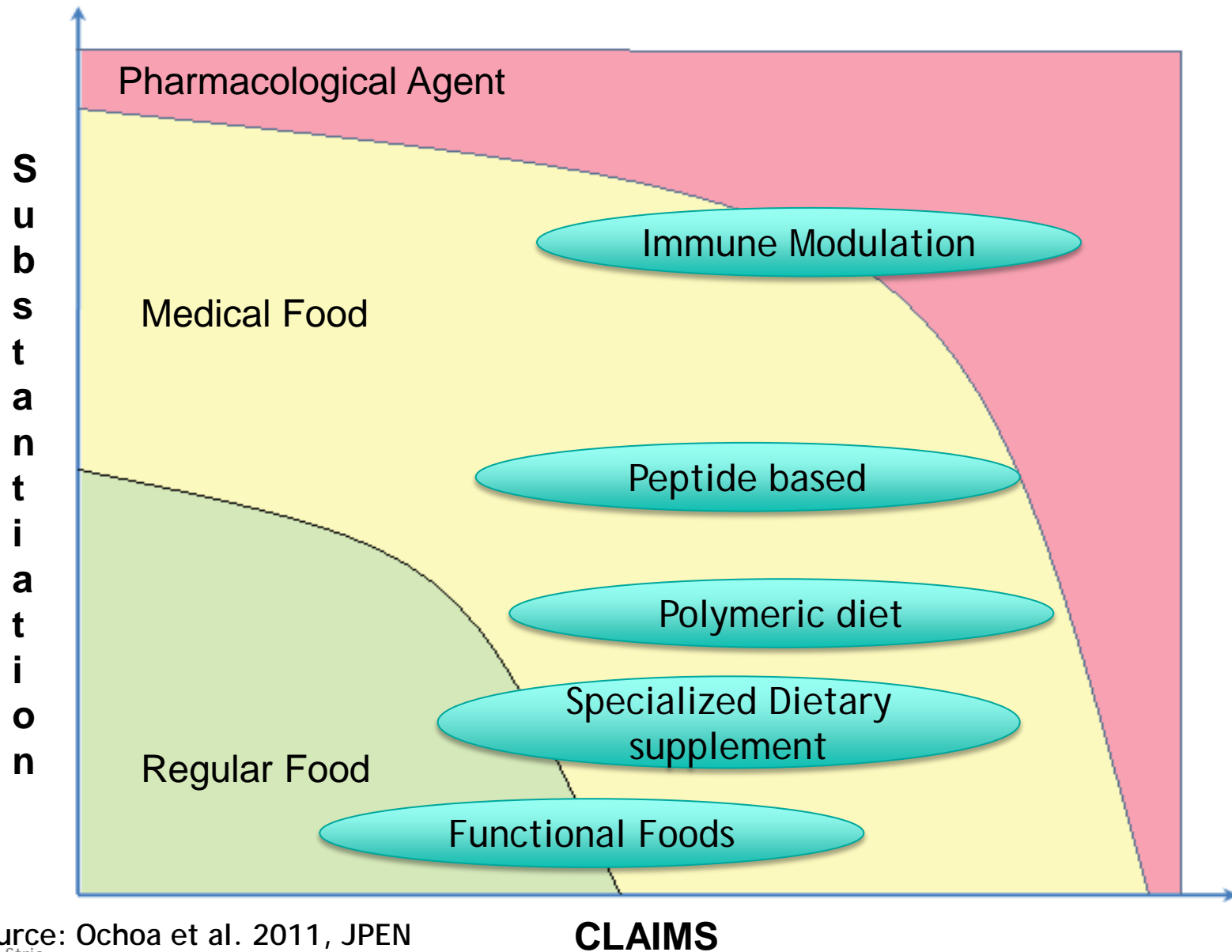
	Prescription Drug	Medical Food	Dietary Supplement
<b>Physician Supervision</b>	Required	Required	Not Required
<b>Pre Market Approval</b>	Yes	No	No
<b>Target Population</b>	Sick/diseases	Sick/disease	Healthy
<b>Claims</b>	As approved by the FDA	Dietary management of nutritional needs	Structure function/maintaining health
<b>Safety</b>	As approved by FDA	GRAS ingredients	Reasonable expectation of safety
<b>Scientific Requirements</b>	Pre-clinical Phase I, II, III	Recognized science & medical evaluation	None/scientific literature/traditional use



# Medical Foods: Few Examples

Brand name	Indication	Promoter
<b>Ultrase MT<sup>®</sup></b>	Exocrine pancreatic insufficiency caused by cystic fibrosis or chronic pancreatitis	Axcan Pharma
<b>Limbrel<sup>™</sup></b>	Nutritional management of metabolic processes associated with osteoarthritis	Primus Pharmaceuticals Inc.
<b>Folgard RX 2.2<sup>®</sup></b>	Management of elevated homocysteine, associated with CVD, stroke, and other diseases	Upsher-Smith
<b>Theramine<sup>™</sup></b>	Dietary Management of Pain and Inflammation	Targeted Medical Pharma LLC
<b>Pulmona<sup>™</sup></b>	Dietary Management of Asthma and Pulmonary Hypertension	Physician Therapeutics LLC
<b>Deplin<sup>™</sup></b>	An adjunctive therapy to antidepressant drugs in treating depression	PamLab

# Requirements for claim substantiation



Source: Ochoa et al. 2011, JPEN

CLAIMS

Stric

# Must read information



A unique space between dietary supplements and pharmaceuticals, the medical foods category may offer novel growth for research-focused companies.

By Dillip Ghosh, PhD, FACN  
*Director, nutriConnect*

In February, Nestlé Health Science, a subsidiary of Nestlé, bought another U.S. medical food company, Covington, LA-based Pamlab, which produces prescription medical foods that support patients with conditions like dementia, diabetic peripheral neuropathy, high-risk pregnancies and

or medical conditions, or during acute phases of illness, injury or disease states.

## FDA on Medical Food

The U.S. FDA designates medical food as a category of substances intended for the clinical dietary management of a particu-

as medical foods are produced under rigid manufacturing practices and maintain high labeling standards.

## Major Health Condition Targets

From a medical standpoint, an increasing number of conditions, such as metabolic

# Market Dynamics

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# Medical Foods: Market Economics

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- ❑ US\$ 10 bn plus in 2010 (Global Industry Analysts, 2011)
- ❑ More than 100 new medical food product launches annually since 2009 (Innova Market Insights, 2011).
- ❑ Combined Medical Foods and Modified Functional Foods market potential is \$38 bn (TCI, 2013)
- ❑ 10% projected market growth (BI, 2012)
- ❑ Double increase in the patent application from 2009 to 2011.

# Medical Foods - An Attractive Segment

## Market Dynamics

- ❑ Increasing incidence of disorders associated with the aging, “Baby Boomer”, population, many of which have inflammation as an underlying cause
- ❑ Increasing burden of costs for governments in most of the developed world associated with the healthcare needs of that aging population
- ❑ Increasing interest, recognition, and acceptance among patients and physicians of the importance of nutrition and other alternative modalities of treatment



# Medical Foods – An Attractive Segment

## Market Need

- Rapidly escalating healthcare costs require new and safer approaches to disease management.
- Disease management without the increasing costs and risk of side effects associated with most prescription drugs.

## Regulatory

- No Pre-market approval required for medical foods due to safety profile resulting from requirement for Generally Recognized As Safe (GRAS) ingredients

# Medical Foods - Increasing Interest

- 2007 Nestle purchased Novartis's nutritional business for \$2.5B
- 2007 Danone acquired Royal Numico and their US medical food business SHS (now Nutricia USA)
- June 2010 Danone acquired Medical Nutrition USA, Inc.
- September 2010 Nestle announced the \$500 million creation of Nestle Health Science, to create an industry bridging the gap between foods and pharmaceuticals
- February 2011 Nestle acquired CM&D Pharma, the company founded, and majority owned by Danilo Casadei Massari, one of the founders of Prismic Pharmaceuticals
- July 2012 Nestle made an equity investment in Accera, the developer of Axona (medical food in development for Alzheimer's disease)
- February 2013 Nestle acquired Pan America Laboratories (Pam Labs)

# Example of the medical foods patents

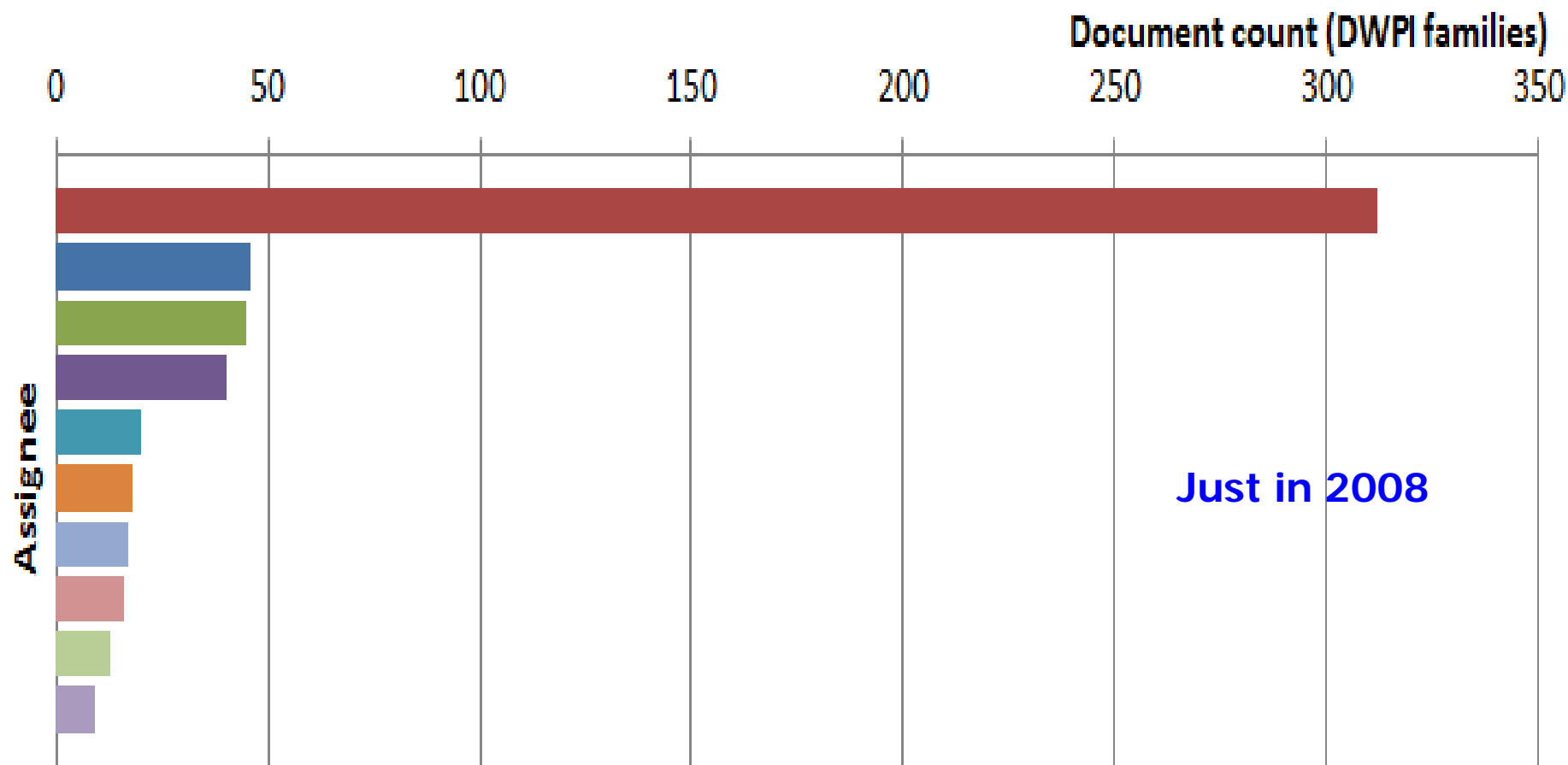
Number	Brief description	Assignee	Filed in other countries
US7514469B2	Formulation for LIMBREL, for Osteoarthritis	Unigen Pharma Inc.	AT, AU, BR, CA, NZ, US, Many more
US7838042B2	Updated formulation for FOSTEUM, for Osteoarthritis	Albion International Inc.	DE, EP, US, WO
US7674482B2	Amino acid based medical foods, targeting neurotransmitters	Targeted Medical Pharma	AU, EP, JP, US, WO
US7943163B2	Method of manufacturing medical foods for Diabetes	Response Scientefic Inc.	US
US6835750B1	Treating Alzheimer's (Axona)	Accera Inc.	AT, AU, BR, CA, US, NZ & many more

Science

Practice

Regulation

## Top patent assignees for keyword search



MONDOBIOTECH LAB AG/MOND-N

DU PONT DE NEMOURS&CO E I/DUPO

SCHWABE GMBH&CO WILLMAR/SCHW-N

MARTEK BIOSCIENCES CORP/MTKB

NESTEC SA/NEST

NUTRICIA NV/DNON

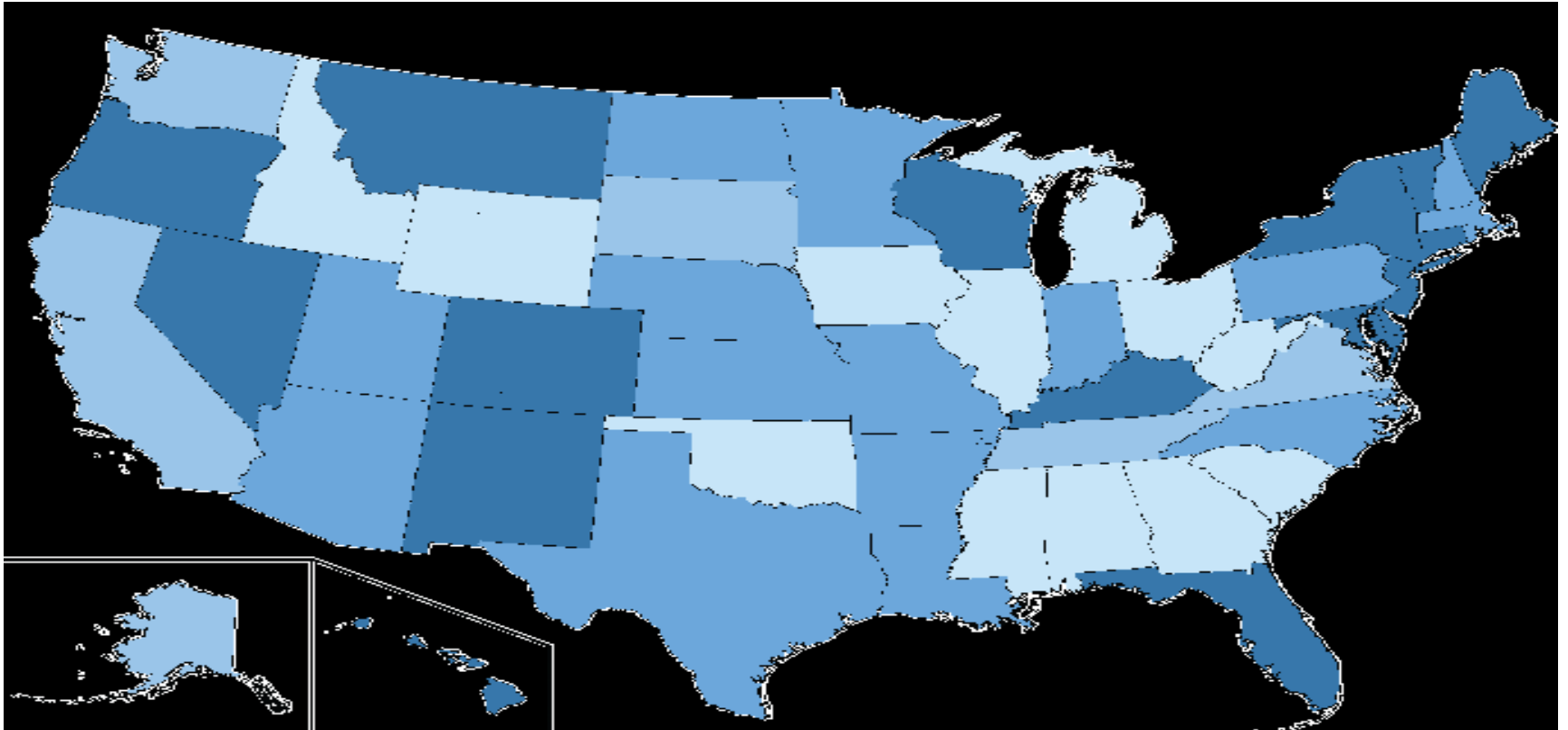
ABBOTT LAB/ABBO

DAMUDE H G/DAMU-I

ZHU Q Q/ZHUQ-I

FONERRA COOP GROUP LTD/FONT-N

# State mandates for medical food coverage in the US



- Select inherited metabolic disorders
- All inherited metabolic disorders

Nature Medicine, 2013

# Challenges

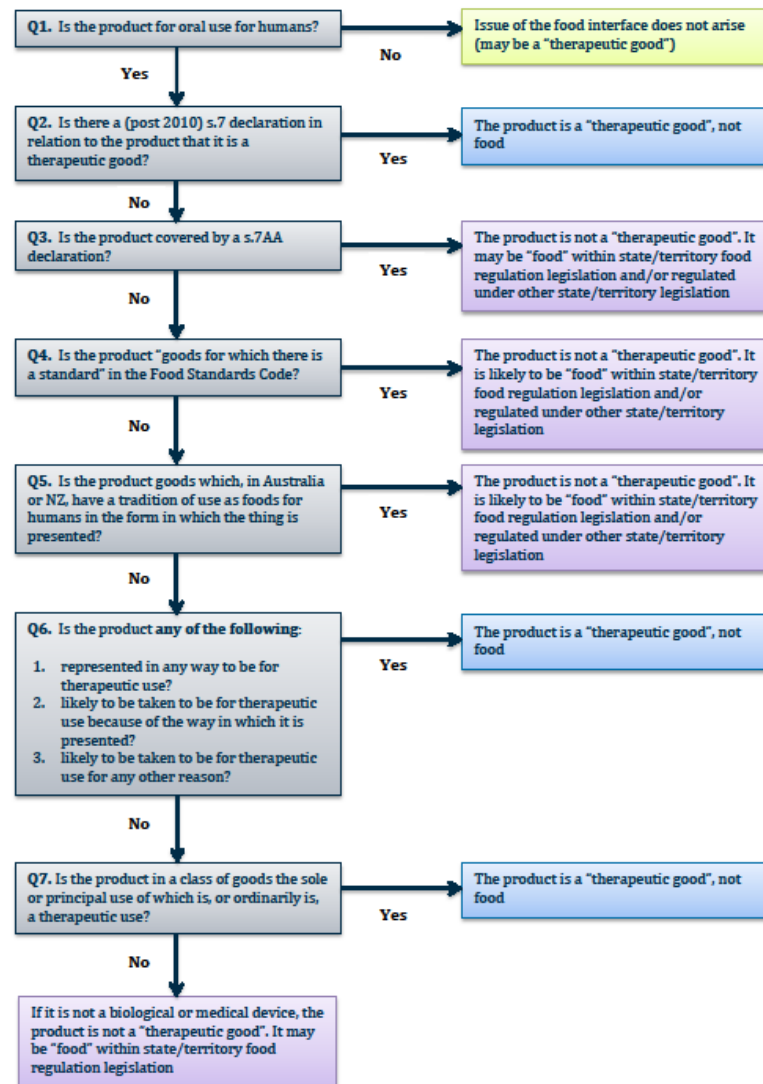
- ❑ Lack of knowledge about medical foods and a high degree of skepticism
- ❑ More product specific clinical research for giving confidence to physicians
- ❑ Regulatory burdens
- ❑ Readily available through pharmacies and through the offices of dispensing physician.
- ❑ Integration in healthcare system & to include into medical insurance scheme





# TGA Guideline

## Food-Medicine Interface Guidance Tool diagram



Food-Medicine Interface Guidance Tool diagram

# Future of Medical Foods

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- ❑ In the US at present, about 45,000 people with Alzheimer's receive the medical food Axona under the supervision of a doctor



- ❑ A three-year-old biotech startup called Ultragenyx Pharmaceutical Inc. has initiated phase 2 drug (UX001) testing for a rare muscle-wasting disorder known as hereditary inclusion body myopathy which defines a new definition of medical food



Transforming good science into great medicine for rare genetic diseases.

# Case Studies

Neurepa

Souvenaid

Axona

# NEUREPA™

A prescription medical food intended for the clinical dietary management of:

- ★ Depression
- ★ Bipolar Disorder
- ★ Schizophrenia



Prismic Pharmaceuticals, Inc.

# NEUREPA™

A proprietary ultra pure Omega-3 triglyceride concentrate, 92% Eicosapentaenoic Acid (EPA), in a 1 gram capsule.

- Prescription medical food intended for the **clinical dietary management** of depression, bipolar disorder, and schizophrenia
- Specifically formulated to provide 92% EPA per gram of oil **at levels not attainable through normal dietary modifications**
- Triglyceride form allows **increased bioavailability** and much reduced “fishy aftertaste”
- **SAFE**. All ingredients in NEUREPA™ are **GRAS** (Generally Recognized As Safe) in accordance with the guidelines established by FDA with side effects no different than placebo
- **Prescription only**. Not available for sale in retail pharmacies or drug stores.

# CNS Program

## Why NEUREPA™

- Studies have clearly demonstrated an association between depression and low dietary intake of Omega-3 fatty acids
- There is clinical evidence suggesting that low levels of Eicosapentaenoic Acid (EPA) in the blood might serve as markers for depression
- Recent genetic analysis has identified risk loci with shared effects among psychiatric conditions including:

Major Depressive Disorder, Bipolar Disorder,  
Schizophrenia



# CNS Program - Clinical Support

## ★ Depression

Omega-3 formulations with high concentrations of EPA ( $\geq 60\%$ ) have been shown to be effective in adjunctive management of patients with depression including those with persistent and treatment resistant depression.

## ★ Bipolar Disorder

Clinical evidence, including randomized trials, support the use of Omega-3 fatty acids (with high concentrations of EPA) in the management of the depressive phase of bipolar disorder.

## ★ Schizophrenia

A number of studies have demonstrated an association between omega-3 fatty acids deficiencies and patients with schizophrenia.

Biochemical studies have shown that levels of Omega-3 fatty acids may be reduced in red blood cell membranes of patients with schizophrenia.



A new and innovative drink for the dietary management of early Alzheimer's disease

Source: <https://www.souvenaid.com.au/>



**Souvenaid®**, a Food for **Special Medical Purposes** for the **dietary management** of the early stages of Alzheimer's disease. & must be used under **medical supervision**

## A new nutritional approach to Alzheimer's disease

Nutritionally supports memory function in the early stages of Alzheimer's disease. Souvenaid® is taken once daily in the early stages of the disease, to nutritionally support the connections in the brain, called synapses.



## Fortasyn™ Connect

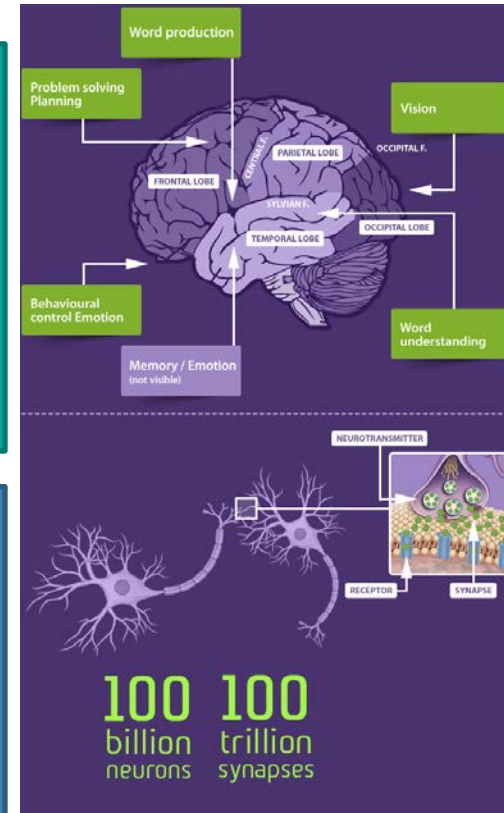
Based on more than 10 years of research and development, Souvenaid® contains a unique combination of nutrients, called Fortasyn™ Connect, which provide the nutritional building blocks that are required by people with early Alzheimer's disease.

# Clinical studies



Two clinical trials have been carried out to look at the effect of Souvenaid® on more than 500 patients with mild Alzheimer's disease and one in mild to moderate Alzheimer's disease.

Souvenaid I trial, investigated the effects of Souvenaid® over a 12 week period. In Souvenir II, the study period was extended to 24 weeks



Scheltens, P et al. Alzheimer's & Dementia. 2010; 6: 1-10.  
Scheltens, P et al. J Alzheimer's disease. 2012; 31: 225-236.  
Shah, et al (2013) Alzheimer's Research & Therapy, 5:59

# Mechanism of action



Alzheimer's disease



Synaptic loss greater than synaptic production in the brain



Multinutrients in Souvenaid® (phosphatide molecules, omega-3 fatty acids, vitamins and antioxidants) help formation and maintenance of synaptic membranes

Source: Company website



# Axona

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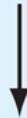
- The product was launched by Accera in 2009
- This is positioned as medical food composed of a proprietary formulation of caprylic acid proposes to target metabolic deficiencies associated with AD



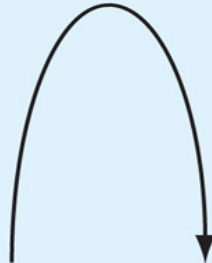
# Mechanism of action



Alzheimer's disease



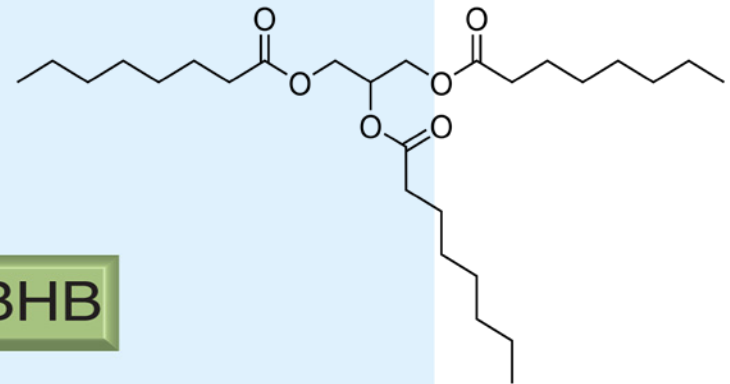
Decrease in brain ability to metabolize glucose,  
the energy resource for the brain



Axona® metabolized to BHB



Provides ketone bodies as energy  
resource for the brain



Source: Company website

# Axona clinical studies

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Axona has been investigated in a clinical study of 152 people with mild to moderate Alzheimer's disease

1. Henderson ST et al. *Nutr Metab (Lond)*. 2009
2. Axona [prescribing information]. Broomfield, CO: Accera, Inc.; November 2012.
3. Geldmacher DS et al. *J Nutr Health Aging*. 2006.

**Claims:** Axona is a prescription **medical food** intended for the clinical dietary management of the metabolic processes associated with mild to moderate Alzheimer's disease.

Source: Company website

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# *Thank you !*

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