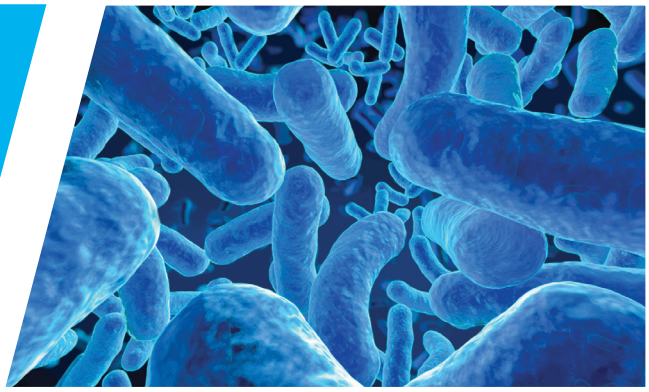
The Development of Live Biotherapeutics

September 24
Rockville, MD



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OPPORTUNITIES AND CHALLENGES IN THE DEVELOPMENT OF LIVE BIOTHERAPEUTICS

A Live Biotherapeutic is a biological product that:

1) contains live microorganisms, such as bacteria or yeast, that are naturally occurring, recombinant, or clonally selected;

2) is applicable to the prevention, treatment, or cure of a disease or condition of human beings; and

3) is not an immunogen-specific vaccine.

While often sharing common origins with probiotics, Live Biotherapeutics are distinct in that they are products developed as therapeutic agents with defined clinical benefit claims. As the characterization of the human microbiome and its link to human health has become better understood, the use of Live Biotherapeutic products in clinical application has shown great promise for reducing infection, stimulating innate immune responses, and modulating gastrointestinal metabolism.

A better understanding of the unique challenges of this therapeutic class will enable more translational research to move forward into new therapeutics.

LEARNING OBJECTIVES

At the conclusion of this meeting, participants should be able to:

- Identify the key considerations for turning clinical research into therapeutic potential
- Discuss the attributes of Live Biotherapeutics that are unique in the development of biological products
- Formulate a list of priority topics and action plans further evaluation

WHO SHOULD ATTEND

- Translational clinicians involved in utilizing probiotics for clinical applications
- Regulators and product developers interested in clarifying regulatory requirements for Live Biotherapeutics
- Sponsors considering the development of commercial Live Biotherapeutics

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TUESDAY, SEPTEMBER 24

7:30-8:30 AM REGISTRATION AND CONTINENTAL BREAKFAST

8:30-8:45 AM WELCOME AND OPENING REMARKS

Norman Baylor
President & CEO
Biologics Consulting Group, Inc.
Former Director of the Office of Vaccines Research and Review (OVRR), CBER, FDA

8:45-9:45 AM SESSION 1, PART I

Translational Research of Live Biotherapeutics

Session Chair

Ryan Ranallo, PhD
Program Officer, Enteric & Hepatic Diseases Branch
NIH

The advances of next generation sequencing have broadened our understanding of microbial ecology and the host-microbe relationship. This combined with the rapid accessibility of bioinformatic and omics based tools is providing a new opportunity to design novel live biotherapeutic (LBP) applications to predict and manipulate microbial community structure and function so as to promote a healthy microbial ecosystem at the level of a single organism, community or the larger host-microbe interface. The improved probability of success with LBP applications requires the integration of genomic standards, translatability of bioinformatic pipelines, and the acceleration of lead optimization processes. This will translate into safer and more effective clinical applications for the prevention and treatment of disease.

Products Based on Food, Food Additives and Dietary Supplements

Robert Merker, PhD
Supervisory Consumer Safety Officer
Division of Biotechnology and GRAS Notice Review
Center for Food Safety and Applied Nutrition, FDA

Commensal, Pathogen Definitions & Human Microbiome

Linda C. Duffy, PhD, MPH
Scientific Chair – trans-NIH Probiotics/Prebiotics and Microbiome Workgroup
NIH Division of Nutrition Research Coordination
HSA Program Director NP - DER
National Center for Complementary and Alternative Medicine
NIH

The Role and Utility of Nonclinical Animal Studies in Safety Evaluation of LBPs

David Pepperl, PhD
Senior Consultant
BCG

9:45-10:15 AM REFRESHMENT BREAK

10:15-11:15 AM SESSION 1, PART II

Translational Research of Live Biotherapeutics (cont)

Ecosystem Therapeutics

Elaine Petrof, MD, MSc
Associate Professor
Dept. Medicine / Infectious Diseases
Gastrointestinal Diseases Research Unit
Queens University & Kingston General Hospital, Canada

Panel Discussion

11:15 AM-12:05 PM SESSION 2, PART I

Product Definition and Characterization

Selection of strains to be included in an LBT should consider source histories to support safety assessment. Establishing methods for strain-specific identity testing will support product control and clinical development. Final identity and potency testing as viable count for products that consist of 2 or more strains may pose challenges. Assurance of purity requires sensitive in-process control and release testing for detection of extraneous contaminants.

Session Chair

Ann Sutton
Affiliate Consultant
Biologics Consulting Group, Inc

Rational Selection of Candidate Bacteria for Bacteriotherapy Development

Trevor Lawley, PhD
Faculty
Wellcome Trust Sanger Institute

Manufacturing Issues in Working with Live Products

John Aunins
Executive Vice President, CMC
Ventures/Seres Health

12:05-1:05 PM LUNCHEON

1:05-2:15 PM SESSION 2, PART II

Product Definition and Characterization (cont)

Quality Control for Potency and Purity

Marian McKee
Principal Scientist
BioReliance

Considerations for Early Product Development of Live Biotherapeutic Products

Cara Fiore, PhD
Master Reviewer, Microbiologist
Division of Vaccines & Related Products Applications
FDA

Panel Discussion

2:15-2:45 PM REFRESHMENT BREAK

2:45-4:45 PM SESSION 3

Planning for Regulatory and Commercial Success

Session Co-chairs

Julienne Vaillancourt, RPh, MPH
Sr Sup Regulatory
Office of Vaccines Research and Review, CBER, FDA

Trent A. Carrier, PhD, MBA
Chief Business Officer
Biologics Consulting Group, Inc.

To foster regulatory and market success, clinical development of a live biotherapeutic (LBP) should focus on demonstrating safety and efficacy in the intended population for use while adhering to principles of good clinical practice (GCP) and human subject protection (HSP). Clinical trials to evaluate LBPs will vary in design, depending on the product, phase of development, and proposed indication. Clinical strategy should be outlined early in product development based on interactions with clinicians, feedback from the FDA and an understanding of key commercial factors.

Clinical Trial Design: A Regulatory Perspective

Jennifer S. Read, MD, MS, MPH, DTM&H
Medical Officer
Division of Vaccines and Related Products Applications,
Office of Vaccines Research and Review
CBER, FDA

Case Study: Results of LBP Phase 1 Studies in Healthy Adults

Taha Keilani, MD
Vice President, Clinical Development
Sigma-Tau Pharmaceuticals, Inc.

Key Factors for Market Success

Maik Klasen, Ph.D.
Managing Director
Adivo Associates

Panel Discussion

4:45-5:00 PM CLOSING REMARKS