CMC Workshop: From Drug Development to Global Supply to Patients

April 15-17, 2013
Washington Marriott Hotel | Washington, DC

PROGRAM CHAIRPERSON

Yasmin de Faria Krim, PharmD, MScRA
Manager, Global CMC Regulatory Affairs
Janssen, Pharmaceutical Companies of
Johnson & Johnson, Belgium

PROGRAM COMMITTEE

Lin-Jau (Christine) Wu Anderson, MS, RAC
Senior Research Scientist
Global Regulatory Affairs
CMC
Eli Lilly and Company, US

Nagesh Bandi, PhD
Senior Manager, Global CMC
Pfizer, US

Ganapathy Mohan, PhD
Executive Director Global CMC Pharmaceutical and Devices, Merck, Sharp and Dohme, Corp., US

Elaine Morefield, PhD
Deputy Office Director, Office of New Drug Quality Assessment, CDER
FDA, US

Peter J. Richardson, PhD
Head of Biologics, Quality of Medicines Sector
European Medicines Agency, UK

Jean-Louis Robert, PhD
Head, Medicines Control Laboratory, National Health Laboratory, Luxembourg

Thirunellai G. Venkateshwaran, PhD
Director, Pharma Technical Regulatory,
Genentech (A member of the Roche Group), US

DIA WORLDWIDE HEADQUARTERS
800 Enterprise Road, Suite 200
Horsham, PA 19044, USA
Tel. +1.215.442.6100 | Fax +1.215.442.6199

WORLDWIDE OFFICES
Basel, Switzerland I Beijing, China I Tokyo, Japan
Mumbai, India I Washington, DC, USA

This workshop, through plenary and parallel breakout sessions, will focus on current challenging topics within the global pharmaceutical/biopharmaceutical arena. Similar to the successful 2011 CMC Workshop, attendees from the different CMC areas will be able to interact with peers from Regulatory Agencies and Industry in sessions enabling cross-functional discussions.

WHO SHOULD ATTEND

Professionals with advanced knowledge of, and experience in:

- CMC Regulatory Affairs
- CMC Writing
- Quality Assurance/Quality Control
- Regulatory Compliance
- API Development and Manufacturing
- Formulation Development and Manufacturing
- Analytical Development
- CMC Life Cycle Management
- CMC Project Management

This program was developed by the CMC Working Group of the DIA Regulatory Affairs Community.

We would like to thank our Media Partner:

IPQ International Pharmaceutical Quality

Co-sponsored by:

aaps American Association of Pharmaceutical Scientists

DIA® www.diahome.org

CONTINUING EDUCATION CREDITS

Drug Information Association has been accredited as an Authorized Provider by the International Association for Continuing Education and Training (IACET), 1760 Old Meadow Road, Suite 500, McLean, VA 22102.

As an IACET Authorized Provider, Drug Information Association offers CEUs for its programs that qualify under the ANSI/IACET Standard. Drug Information Association is authorized by IACET to offer 1.7 CEUs for this program. Participants must attend the entire program in order to be able to receive an IACET statement of credit. No partial credit will be awarded.

If you would like to receive a statement of credit, you must attend the program, sign-in at the DIA registration desk each day of the program, and complete the on-line credit request process through My Transcript. To access My Transcript, please go to www.diahome.org, select ìLogin to My DIAî and you will be prompted for your user ID and password. Select ìMy Transcriptî (left side bar) and ìCredit Requestî to process your credit request. Participants will be able to download a statement of credit upon successful submission of the credit request. My Transcript will be available for credit requests on Wednesday, May 1, 2013.

Disclosure Policy

It is Drug Information Association policy that anyone in a position to control the content of a continuing education activity must disclose to the program audience (1) any real or apparent conflict(s) of interest related to the content of their presentation and/or the educational activity, and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Faculty disclosures will be included in the course materials.

LEARNING OBJECTIVES

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

- Discuss the current CMC hot topics for chemical and biological products in a globalized environment
- Explain current issues related to the pharmaceutical supply chain
- Describe the international initiatives in the area of impurities
- Discuss how to develop CMC documentation for clinical trials
- Explain trends for biorelevant specifications and bioequivalence studies
- Discuss continuous manufacturing, control strategy, and process validation
- Indicate the recent trends for specific types of products
- Recognize how to adhere to post-approval change management protocols
- State updates in the area of stability

DIA'S CERTIFICATE PROGRAM

This program is part of DIA's Certificate Program and is awarded the following:

- Regulatory Affairs Certificate Program: 10 Elective Units

For more information go to ***diahome.org/certificateprograms***

DAY 1 | MONDAY, APRIL 15

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

8:30 – 8:45 AM WELCOME AND INTRODUCTION

PROGRAM CHAIRPERSON:

Yasmin de Faria Krim, PharmD, MScRA
Manager, Global CMC Regulatory Affairs
Janssen, Pharmaceutical Companies of Johnson&Johnson, Belgium

8:45 – 10:15 AM SESSION 1

Globalization in the CMC Area - Collaboration between Regulatory Agencies

SESSION CHAIR:

Jean-Louis Robert, PhD
Head, Medicines Control Laboratory
National Health Laboratory, Luxembourg

From the hurdles of getting global regulatory approvals through updates on the EMA/FDA parallel assessment pilot program for QbD submissions or global activities for GMP inspection, this session will focus on globalization of activities in the area of both CMC assessment and GMP inspections. Current and future challenges as well as opportunities to facilitate global approvals of drugs will be addressed through feedback from representatives from both Industry and Agencies. A Question/Answer session will provide attendees with the opportunity to comment and share their thoughts on current initiatives.

Challenges in Getting Global Regulatory Approvals

Ganapathy Mohan, PhD
Executive Director Global CMC Pharmaceutical and Devices, Merck, Sharp and Dohme, Corp., US

Lessons Learned Developing a Global QbD Pilot for Biologics

Lynne Krummen, PhD
Senior Director, Global Head – Biologics, Pharma Technical Regulatory Affairs
Genentech (A member of the Roche Group), US

FDA Update on Inspection Globalization Activities

Diana Amador Toro, PhD
Director, New Jersey District, Office of Regulatory Affairs
FDA, US

Q&A Panel

All Speakers Above and

Elaine Morefield, PhD
Deputy Office Director, Office of New Drug Quality Assessment, CDER
FDA, US

10:15 – 10:45 AM REFRESHMENT BREAK

10:45 AM – 12:15 PM SESSION 2

TRACK A

Challenges and Opportunities for the API supply chain following ICH Q11

Session Chair:

Timothy J.N. Watson, PhD
Research Fellow, GCMC Advisory Office
Pfizer, US

This session will feature speakers willing to expand upon some of the more challenging issues for the API supply chain as they relate to ICH Q11 (Development and Manufacture of Drug Substances). ICH Q11 provides opportunities for improving regulatory global harmonization expectations for dug substance development and manufacture. However, implementation of ICH Q11 may not necessarily align with region to region interpretations and/or current regulations. For example, the interpretation and assessment of criticality of quality attributes and process parameters, the justification of a design space based on small-scale models, the development of a holistic control strategy, and the role of post-approval process verification have varied interpretations form both industry and regulatory authorities.

Impact of Control Strategy on API — the ICH Q11 View

Betsy Fritschel, MS
Director, Regulatory Intelligence, Quality and Compliance Worldwide
Johnson & Johnson, US

Control strategy: developing, interpreting, and sharing the data rich content of an enhanced submission

Kevin Seibert, PhD, MS
Senior Research Advisor, Chemical Product R & D
Eli Lilly and Co., US

Design Space — The challenges of balancing knowledge, risk, and uncertainty

John V. Lepore, PhD
Senior Director, Chemical Process Development and Commercialization
Merck & Co., Inc., US

TRACK B

Pharmacopeias: Collaboration Across Borders

Session Chair:

Erin Gu Wang, MS, RAC
Associate Consultant – Compendial Affairs, Global Quality Laboratory
Eli Lilly and Company, US

Pharmacopeias are recognized sources of public standards for pharmaceutical products, ingredients, components, and agreed common practices. Compendial standards are typically comprised of test procedures and associated acceptance criteria. These standards are enforced by regulatory agencies, and are quality standards to be met by all manufacturers/applicants for certain territories. Companies that wish to market products globally must have a robust strategy for meeting compendial requirements for each market. Collaboration between regulators, industry, and pharmacopeias is crucial to ensure that these standards serve to benefit public health, both regionally and internationally. This session will provide an overview of the United States and European compendial science activities. The industry presentation will share experiences in managing public standard implementation globally. Panel discussion will allow participants to have deeper interactions with the speakers and the fellow participants.

USP Compendial Activities

Shawn Dressman, PhD
Vice President, Chemical Medicines, USP-NF
United States Pharmacopeia, US

European Pharmacopoeia Overview

Michael Wierer, PhD
Deputy Head, European Pharmacopoeia Department
European Directorate for the Quality of Medicines and HealthCare, France
Presentation will be delivered via webinar

Compendial Affairs

Craig Hamilton, PhD
Director-Operational Excellence, Global Quality Laboratory
Eli Lilly and Company, US

Erin Wang, MS, RAC
Associate Consultant – Compendial Affairs, Global Quality Laboratory
Eli Lilly and Company, US

TRACK C

Clinical Trials and Marketing Applications in Latin America CMC Considerations

Session Chair:

Bekki E. Komas, MS
Director, CMC Advocacy
GlaxoSmithKline, US

ANVISA regulators will discuss the CMC and Clinical Trials requirements in the Latin America region. Industry speakers will share opportunities, challenges and recent experience with clinical trial and marketing applications. This will include experience with recent guidance such as the API Stability Guidance Resolution 45 issued August 2012. This will be followed by a panel discussion including additional industry experts. An opportunity for the audience to master the intricacies of CMC aspects of clinical trial and marketing applications in the Latin America market with special focus on Brazil.

CMC Requirements in Brazil - Regulatory Aspects

Ana Carolina Araujo, PharmD
Sanitary Surveillance and Regulation Specialist – Pharmacist, Post-Approval Coordination Group (COPRE), Pharmaceutical Technology General Office (GTFAR)
Anvisa, Brazil

Clinical Trial Requirements in Brazil – CMC Considerations

Fanny Viana, PharmD
Sanitary Surveillance and Regulation Specialist – Pharmacist, Research and Clinical Trials Coordination Group (COPEM), Safety and Efficacy General Office (GESEF)
Anvisa, Brazil

Industry Experience with Clinical Trial and Marketing Applications In Latin America

Bekki E. Komas, MS
Director, CMC Advocacy
GlaxoSmithKline, US

Panel Industry Representative

Ganapathy Mohan, PhD
Executive Director Global CMC Pharmaceutical and Devices, Merck, Sharp and Dohme, Corp., US

Marcio Silva, PharmD
Regulatory Manager for GSK Brazil
GlaxoSmithKline Brazil Ltda, Brazil

12:15 – 1:30 PM LUNCH

1:30 – 3:00 PM SESSION 3

TRACK A

Global Supply Chain: Counterfeits

Session Chair:

Ganapathy Mohan, PhD
Executive Director Global CMC Pharmaceutical and Devices, Merck, Sharp and Dohme, Corp., US

In this session, the speakers will focus on counterfeits in the pharmaceutical global supply chain, their impact and initiatives to counter this continuously growing problem. Strategies to stay ahead of counterfeits are definitively a challenge. Initiatives taken in the area of packaging to prevent counterfeiting through innovative techniques have shown to be promising however more and more fighting counterfeits in the supply chain will rely on advanced technologies

Assurance of Quality in the Global Supply Chain

Jennifer Finnegan McCafferty, PhD
Vice President, External Quality, Global Manufacturing & Supply
GlaxoSmithKline, US

Global Supply Chain Security with Focus on EU Falsified Medicine Directive

Michael Rose, MSE
Vice President, Supply Chain Visibility
Johnson & Johnson Health Care Systems, Inc.

TRACK B

Impurities – Genotoxic Impurities

Session Chair:

Stephen Miller, PhD
CMC-Lead, Office of New Drug Quality Assessment
CDER, FDA, US

Genotoxic compounds may be used as synthetic intermediates, reagents, etc, or may be formed as by-products during the manufacture of pharmaceuticals. Maintaining any genotoxic impurities at or below levels that are acceptable presents challenges to industry and to regulators due to the need for highly sensitive analytical methods, and the diversity of structures and reactivity. This session will present current approaches under consideration for an ICH guideline, and discuss experience from both the industry and regulatory perspectives on the control of genotoxic impurities in pharmaceuticals.

Genotoxic Impurities: EU Experience and Comparison with the Current Discussion at ICH

Jean-Louis Robert, PhD
Head, Medicines Control Laboratory
National Health Laboratory,
Luxembourg

Genotoxic Impurities - An Industry Perspective

Kate Arnot, MSc
CMC Director, Global Regulatory Affairs
AstraZeneca, UK

Genotoxic Impurities as Critical Quality Attributes

Stephen Miller, PhD
CMC-Lead, Office of New Drug Quality Assessment
CDER, FDA, US

TRACK C

Clinical Trials in the European Union (Biologicals)

Session Chair:

Jilla Boulas, MS
Director
Voisin Consulting, Inc., US

This session will focus on the EU guideline for CMC requirements for clinical trials for Biologicals, released in May 2012. Experiences and perspectives will be shared on the key elements when compiling and maintaining CMC documentation for investigational medicinal products. An update on the proposed EU Clinical Trials Regulation and its opportunities for the future and Biologicals in particular will also be discussed: simplified submission, authorization procedures, etc.

An Industry View Point On The CMC Requirements For Clinical Trials In The EU (Biotherapeutics)

Stefanie Pluschkell, PhD
Senior Director, Global Biotherapeutics, Policy and New Product Development
Pfizer Inc., US

CMC Aspects Of Clinical Trial Applications: What The Regulators Ask *An Industry Perspective*

Khandan Baradaran, PhD
Associate Director, Regulatory Affairs
Biogen Idec, US

3:00 – 3:30 PM REFRESHMENT BREAK

3:30 – 5:30 PM SESSION 4

TRACK A

Drug Shortages

Session Chair:

Norman R. Schmuff, PhD
Associate Director for Product Quality
CDER, FDA, US

Drug and other medical product shortages have the potential to adversely affect patient care by delaying treatment or forcing the use of second-choice products. Some recent shortages have involved drugs for life-threatening conditions and, in some cases, the product in shortage has been the only product for the patient's condition. This is a significant public health problem, one that deserves the concerted attention of government and industry. The problem peaked in 2011 when 251 drugs were declared in short supply In 2012 this number declined to approximately 150, according to current estimates. In this session causes, consequences, and FDA action to address and prevent drug shortages will be discussed.

Current Trends in Drug Shortages — A University of Utah Drug Information Perspective

Erin Fox, PharmD
Director, Drug Information Service
University of Utah Hospitals & Clinics, US

FDA's Role in Mitigating Drug Shortages

CAPT Valerie Jensen, RPh
Associate Director, CDER Drug Shortage Staff
FDA, US

Economic and Technological Drivers of Generic Sterile Injectable Drug Shortages

Marta Wosinska, PhD
Senior Economic Advisor
FDA, US

TRACK B

Impurities – Metal Impurities

Session Chair:

John F. Kauffman, PhD, MBA
Research Chemist, Division of Pharmaceutical Analysis, Office of Pharmaceutical Science, CDER
FDA, US

This session will provide a status update on the ICH Q3D guideline on metal impurities in pharmaceuticals and a description of the IPEC Q3D Information Exchange Request documents. In addition, examples of risk-based approaches to controls on metal impurities in pharmaceuticals will be described. At the conclusion of the presentations, the panel of speakers will be available to answer questions from the audience.

Approaches To Assess And Control Metal Impurities In Drug Products

Mark G. Schweitzer, PhD
Director, NCE Analytical R&D LC
AbbVie, Inc., US

ICH Q3D Metal Impurities – Excipient Realities and Challenges

Katherine Ulman
Associate Scientist & Global Regulatory Compliance Manager
Dow Corning Healthcare, US

Metal Analysis in Pharmaceuticals: Impending Changes and Potential Strategies to Address Them

Nancy Lewen
Principal Scientist
Bristol-Myers Squibb, US

TRACK C

Clinical Trials Submissions in Asia Pacific Region

Session Chair:

Lin-Jau (Christine) Wu Anderson, MS, RAC
Senior Research Scientist, Global Regulatory Affairs
CMC
Eli Lilly and Company, US

There are many new regulatory developments in the Asia Pacific region. In order to have a successful clinical trial application in this region, the sponsors need to stay on top of the changes and new requirements. This session will cover the most recent hot topics in China, South Korea, India, Taiwan, Hong Kong, Malaysia, etc., regarding the clinical trial submission.

Clinical Trial Application in Asia Pacific Region other than China

Xiling Song, MS
Regulatory Product Manager, Pharma Technical Regulatory
Genentech, Inc., US

Clinical Trial Application in China

Chi-Wan Chen, PhD
Executive Director, Global CMC, Global Research & Development, Pfizer Inc., US

Key Regulatory Development in China

Min Gui, PhD
Director, CMC Asia Pacific, China CMC, Global Regulatory Sciences
Bristol-Myers Squibb, China

5:30 PM END OF DAY 1

5:30 – 6:30 PM NETWORKING RECEPTION

DAY 2 – TUESDAY, APRIL 16

7:00 – 8:00 AM REGISTRATION AND CONTINENTAL BREAKFAST

8:00 – 9:30 AM SESSION 5

Clinically Relevant Specifications

Session Chair:

Nagesh Bandi, PhD
Senior Manager, Global CMC
Pfizer, US

Due to the critical role that dissolution plays in the bioavailability of the drug, in vitro dissolution can serve as a relevant predictor of the in vivo performance of the drug product. This plenary session will discuss relevant factors that should be considered in developing a clinically relevant dissolution method and specifications. The role of dissolution in the development of the appropriate design space will also be illustrated. Establishing the appropriate dissolution specifications will assure that the manufacture of the dosage form is consistent and successful throughout the product's life cycle and that each dosage unit within a batch will have the same pharmaceutical qualities that correspond to those shown to have an adequate safety and efficacy profile.

Presentation

Richard T. Lostritto, PhD, MS
Associate Director, Office of New Drug Quality Assessment, DPAMS
CDER, FDA, US

How Do We Achieve Clinical Relevancy And Is This A Regulatory Requirement?

Ganapathy Mohan, PhD
Executive Director Global CMC Pharmaceutical and Devices, Merck, Sharp and Dohme, Corp., US

Challenges for Developing Dissolution Methods with Clinically Relevant Specifications

Roy De Maesschalck, PhD
Associate Director, Dissolution testing
Janssen Pharmaceutical Companies of Johnson and Johnson, Belgium

9:30 – 10:00 AM REFRESHMENT BREAK

10:00 AM – 12:00 PM SESSION 6

TRACK A

Regional Approaches to Bioequivalence

Session Chair:

Anther (Chi) Keung
Director, Clinical Pharmacology Leader
Janssen, Pharmaceutical Companies of Johnson&Johnson, US

Bioequivalence (BE) studies are typically performed following CMC changes either as bridge studies between clinical trial and to-be-marketed formulations or during lifecycle management (post-approval changes). In addition, BE studies are also performed to support Biowaiver strategy for combination products. The design, performance, and evaluation of bioequivalence studies have received major attention lately. This session proposes to focus on the difference in bioequivalence studies requirements across regions/countries based on the more general and global requirements.

The WHO approach to Bioequivalence: the global perspective

Jan Welink
Senior Pharmacokinetic Assessor
Medicines Evaluation Board, Netherlands

Brazilian (Anvisa) Requirements for Bioequivalence Studies

Márcia Sayuri Takamatsu Freitas, MSc
Partner - Owner
BE Consulting, Brazil

Japan (PMDA) Requirements for Bioequivalence Studies

Vijay Tammara, PhD
Vice President, Regulatory Affairs
Nuron Biotech Inc., US

TRACK B

Control Strategy - Lessons Learned

Session Chair:

Elaine Morefield, PhD
Deputy Office Director, Office of New Drug Quality Assessment, CDER
FDA, US

This session will cover industry and regulator experiences with implementing integrated control strategies for small molecule and biologic products. Control strategy discussion may include process controls, in process tests, design space and real time release testing. Drug substance and drug product considerations will be covered. Parenteral and solid oral dosage form examples will be presented.

Regulatory Considerations for Control Strategy

Elaine Morefield, PhD
Deputy Office Director, Office of New Drug Quality Assessment, CDER
FDA, US

Experiences in Establishing a Commercial Control Strategy for a Small Molecule Product

John Groskoph, MBA
Sr. Director, New Products – CMC, Global CMC
Pfizer, US

Considerations for Establishing a Control Strategy for a Biologic Product Throughout Development

Allison J. Wolf, MS
Research Scientist, Global Regulatory Affairs CM&C
Eli Lilly and Company, US

TRACK C

Antibody-Drug Conjugates (ADC)

Session Chairs:

Thirunellai G. Venkateshwaran, PhD
Director, Pharma Technical Regulatory, Genentech (A member of the Roche Group), US

Sarah Pope Miksinski, PhD
Acting Director, Division 1, Office of New Drug Quality Assessment
CDER, FDA, US

The goal of this session is to discuss the current status of the various control systems for Antibody-drug conjugates (ADCs) and some of the typical challenges that are encountered during their development and commercialization. ADCs, monoclonal antibodies (MAbs) coupled to cytotoxic agents, utilize the specificity of a monoclonal antibody to deliver a cytotoxic drug to tumor cells. Since an ADC product is a mixture of conjugated species, appropriate assays are key to characterize and release ADC products as well as identifying potential critical quality attributes (CQAs) for the conjugates, including those for the antibody, the cytotoxic agent, and the linker.

Antibody Drug Conjugates – Current Status of Regulations

Debasis Ghosh
Office of New Drug Quality Assessment
CDER, FDA, US

Marjorie Shapiro, PhD
Chief of the Laboratory of Molecular and Developmental Immunology, OBP
CDER, FDA, US

Analytical Characterization and Control Strategies for Antibody Drug Conjugates

Heyi Li, PhD
Senior Principal Scientist
Analytical R&D, BioTx PS; Pfizer, Inc., US

12:00 – 1:00 PM LUNCH

1:00 – 3:00 PM SESSION 7

TRACK A

Pediatrics: Challenges and Specific Requirements

Session Chair:

Jenny Walsh
Director
Jenny Walsh Consulting Ltd., UK

The development of paediatric medicines can be particularly challenging, since it is necessary to consider the diversity of this patient population in terms of physiological and biological maturation, compliance challenges such as acceptable palatability and potential safety concerns associated with the required excipients. This session will discuss some of these challenges in more detail and provide an overview of the European perspective including Paediatric Investigation Plans (PIPs). An update on the joint AAPS/Pediatric Task Force Points to Consider Document will also be provided.

Challenges Associated with Developing Medicines for Children - An Industry Perspective

Gossett Campbell, PhD, CQPA
Scientific Investigator, Formulation & Process Analysis
GlaxoSmithKline, US

EU Paediatric Investigation Plans

Mike Saleh, MSc
Director, Global Chemistry, Manufacturing and Controls
Pfizer, Inc.

AAPS/Pediatric Task Force Points to Consider Paper on Pediatric Formulations Development

Georgia Charkoftaki, PhD
Research Associate Laboratory of Biopharmaceutics and Pharmacokinetics National and Kapodistrian University of Athens, Faculty of Pharmacy, Greece

TRACK B

Control Strategy in Continuous Manufacturing

Session Chair:

Thirunellai G. Venkateshwaran, PhD
Director
Pharma Technical Regulatory, Genentech, (a member of the Roche group), US

Continuous manufacturing is the ability to process materials to a defined end state without any process interruptions. Continuous processes while prevalent in industries such as petrochemical manufacturing are a fairly innovative concept in the Pharmaceutical Industry. It is often seen as the next step in the cGMP's for the 21st century initiative and is currently being explored by a number of companies. The advantages of continuous processing while many are also accompanied by some uncertainties. The purpose of this session is to focus on continuous manufacturing process and control strategies for continuous processing. The challenges as well as opportunities of continuous manufacturing will be discussed as part of the session.

Continuous Processing – A Regulatory Perspective

Sharmista Chatterjee, PhD
Chemist, Office of New Drug Quality Assurance
CDER, FDA, US

Implementation of Continuous Manufacturing in the Pharmaceutical Industry: Challenges and Opportunities

Nirdosh Jagota, PhD
Vice President and Global Head - Small Molecules
Genentech (A member of the Roche Group), US

Continuous Manufacturing: Is it Feasible or Not?

James Evans, PhD
Associate Director, Novartis-MIT Center for Continuous Manufacturing
Massachusetts Institute of Technology, US

TRACK C

Biosimilars

Session Chair:

Gregory C. Davis, PhD
Consultant
Davis Consulting Services

This session will examine the EU, Canada, and US regulatory pathways for the registration of biosimilars and discuss lessons learned from the implementation and application of these pathways to product reviews. The speakers will also update any anticipated changes to the pathways or to existing guidance.

EU Regulatory Update On Guidance And Experience With Biosimilars

Peter J. Richardson, PhD
Head of Biologics, Quality of Medicines Sector
European Medicines Agency, UK
Presentation will be given remotely via webinar

Canadian Approach to the Regulation of Subsequent Entry Biologics (Biosimilars)

Anthony Ridgway, PhD
Senior Regulatory Scientist
Office of the Director, Centre for Evaluation of Radiopharmaceuticals and Biotherapeutics, Biologics and Genetic Therapies Directorate, Health Products and Food Branch, Canada

FDA Update on the Implementation of the Biosimilar Approval Pathway

Steven Kozlowski, MD
Director of the Office of Biotechnology Products
CDER, FDA, US

3:00 – 3:30 PM REFRESHMENT BREAK

3:30 – 5:00 PM **SESSION 8**

New Technologies

Session Chair:

Fernando J. Muzzio, PhD
Director, NSF ERC on Structured Organic Particulate Systems
Professor II, Chemical and Biochemical Engineering, Rutgers University
US

New technologies are rapidly gaining momentum in pharmaceutical manufacturing including new manufacturing and mathematical modeling approaches. Continuous manufacturing methods have attracted enormous interest in recent years. In this and in other areas, a growing toolbox of particle engineering methodologies are enabling both more effective manufacturing (by allowing the user to design particles with tailored properties) and more accurate drug release profiles (by allowing tighter control of particle size, particle-substrate interactions, etc). To help support these and other technologies, sophisticated modeling approaches have evolved. In this session, we will review recent advances from both a technological and regulatory perspective.

New Technologies For Continuous Manufacturing

Fernando J. Muzzio, PhD
Director, NSF ERC on Structured Organic Particulate Systems
Professor II, Chemical and Biochemical Engineering,
Rutgers University
CSO and co-founder, Acumen Biopharma, US

Mathematical Models for Enhanced Control of Pharmaceutical Manufacturing

Christine Moore, PhD
Director, Office of New Drug Quality Assessment
CDER, FDA, US

New Particle Engineering Technologies For Modified Release Applications

Raj Dave, PhD
Distinguished Professor of Chemical, Biological and Pharmaceutical Engineering
New Jersey Institute of Technology, US

5:00 PM **END OF DAY 2**

DAY 3 – WEDNESDAY, APRIL 17

7:00 – 8:00 AM REGISTRATION AND CONTINENTAL BREAKFAST

8:00 – 9:30 AM SESSION 9

TRACK A

Innovative Approaches for Stability Programs

Session Chair:

Kenneth C. Waterman, PhD
President, FreeThink Technologies, Inc., US

Stability methodologies for drug substances and drug products continue to evolve both from a scientific and regulatory perspective. This session will explore advances in scientific understanding of stability using the Accelerated Stability Assessment Program (ASAP) and how it impacts decisions in drug development. The session will also explore the changing ways that pharmaceutical companies look to meet their regulatory and ethical commitments while reducing low-value and often time-consuming testing.

Global Acceptance Of Lean Stability Strategies: Still Just A Pipe-Dream Or Are We Making Real Progress?

Stephen Colgan, PhD
Senior Director
Pfizer Inc., US

Using Scientific Methods To Model Shelf-Life For Solid Drug Substances And Drug Products

Kenneth C. Waterman, PhD
President, FreeThink Technologies, Inc., US

Using Predictive Stability To Enhance Product Knowledge

Zhixin Jessica Tan, PhD
Principal scientist
Amgen Inc., US

TRACK B

Post-approval Change Management Protocols

Session Chair:

Moheb M. Nasr, PhD
Vice President, CMC Regulatory Strategy
GlaxoSmithKline, US

A post-approval change management protocol describes changes that a company would like to implement during the lifecycle of the product and how these would be prepared and verified. The use of post-approval protocols can greatly facilitate life-cycle management of CMC. However, the utilization of post-approval management protocols has not been widely embraced by industry. Additional experience and regulatory clarity could speed up implementation.

Leading regulators, from Europe and United States, and industry speakers will share their perspectives on the utilization of post-approval management protocols, outline implementation challenges and provide suggestions to facilitate preparation and approval. Presentations will be followed by panel discussions

Regulatory Consideration For The Development And Filing Of Post-Approval Management Protocols – EU Perspective

Jean-Louis Robert, PhD
Head, Medicines Control Laboratory
National Health Laboratory, Luxembourg

Regulatory Consideration For The Development And Filing Of Post-Approval Management Protocols – FDA Perspective

Christine Moore, PhD
Director, Office of New Drug Quality Assessment
CDER, FDA, US

Utilization Of Post-Approval Management Protocols In The Implementation Of Quality By Design (Qbd) For Biological Drug Products

Lynne Krummen, PhD
Vice President, Global Head – Biologics, Pharma Technical Regulatory Affairs
Genentech (A member of the Roche Group), US

TRACK C

Advanced-Therapy Medicinal Products (ATMP)

Session Chair:

Deborah A. Hursh, PhD
Senior Investigator
CBER, FDA, US

Interest in ATMP's continues to grow, with the recent authorization of Glybera in the EU and there are a substantial number of products under development. The Committee for Advanced Therapies at the EMA has received a large number of scientific advice requests and FDA has seen increasing numbers of Investigational New Drug Applications and more advanced Phase trials. This experience shows that common issues arise for CMC related to characterization, consistency and comparability, particularly for potency assays. The current regulatory climate will be explored and examples discussed to highlight these aspects.

CMC Regulatory Considerations for Advanced Therapies

Deborah A. Hursh, PhD
Senior Investigator
CBER, FDA, US

Development Experience Gained From The EU Authorisation Glybera

Harald Petry, PhD
VP, Director Research and Development
uniQure B.V, Netherlands

Presentation

Robert A. Preti, PhD
President and Chief Scientific Officer
PCT Cell Therapy Services, US

9:30 – 9:45 AM REFRESHMENT BREAK

9:45 – 11:15 AM SESSION 10

TRACK A

Shipments Monitoring and Temperature Control

Session Chair:

Kenneth C. Waterman, PhD
President, FreeThink Technologies, Inc., US

While storage and shipping conditions are qualified based on stability studies, invariably in an increasingly dynamic world market, materials will experience excursions. The responsible company needs to justify the acceptability (or make a decision to discard) of any such drug products. This session will discuss the scientific and regulatory basis for determining the appropriate course of action.

Storage Temperature × Humidity Design Space And Logistics Excursions

William R. Porter, PhD
Principal Scientist
Peak Process Performance Partners, US

Dealing With Temperature Excursions During Shipment And Storage

Manuel Zahn, PhD
Managing Director
3R Pharma Consulting GmbH, Germany

Use Of Stability Modeling As Part Of Qbd To Determine The Impact Of Excursions On Drug Substances And Drug Products

Kenneth C. Waterman, PhD
President, FreeThink Technologies, Inc., US

TRACK B

Process Validation: Maintaining the State of Validation in the Continuous Improvement Paradigm

Session Chair:

Prabu Nambiar, PhD, MBA, RAC
Principal, Syner-G Pharma CMC Consulting, LLC, US

Continuous improvement (CI) is one of the key benefits of drug development under the QbD based drug development paradigm. The traditional process validation requirements might be interpreted to be limiting in terms of the industry's ability to realize the full benefits of the CI process. In this regard, FDA and EMA have issued a draft process validation guidance document that provide for the possibility of continuous process verification (CPV) to cover an alternative approach to process validation based on a continuous monitoring of manufacturing performance. The FDA draft guidance has focused on process validation as a three stage process – Process Design, Process Qualification and Continuous Process Verification. The objective of this session is to explore how companies can use the CPV approach to maintain the state of validation and be in full compliance with regulatory requirements.

Continuous Improvement and Continuous Process Verification: FDA Perspectives

Kelli Dobilas
Office of Regulatory Affairs
FDA, US

Maintaining the State of Validation in an Outsourced Manufacturing Paradigm

Eda Ross Montgomery, PhD
Senior Director
Shire Pharmaceuticals, US

Innovative Approaches To Process Validation

Thirunellai G. Venkateshwaran, PhD
Director, Pharma Technical Regulatory, Genentech (A member of the Roche Group), US

TRACK C

Drug/Device Combination Products

Session Chair:

Douglas Mead
Director, CMC Global Regulatory Affairs
Janssen Pharmaceutical Companies of Johnson&Johnson, US

Evolving regulations and requirements related to drug/device combination products have increased the complexity of their development, including questions of their use in clinical trials, design validation, regulatory submission content, and post market changes. This session will focus on the latest changes and trends in regulatory requirements that are impacting the introduction of new drug delivery technologies. In addition to increasingly specific technical requirements, health authorities are also starting to expect that to-be-marketed presentations be used in clinical trials and that the usability of these devices be assessed in formal human factors studies. Session speakers will address the current regulatory landscape for these requirements and strategies that may successfully meet them.

FDA Updates to Combination Product Regulations

Patricia Y. Love, MD, MBA
Deputy Director, Office of Combination Products
CDER, FDA, US

Human Factors Study Requirements For Drug/Device Combination Products

Mark A. Marley
Principal Research Scientist – Regulatory-Devices
Eli Lilly and Company, US

Clinical Trial Challenges Related To Drug/Device Combination Products

Donna French, PhD
Senior Director
Genentech, (a member of the Roche group), US

Le Dao
Principle Research Associate
Genentech, (a member of the Roche group), US

11:15 – 11:30 AM BREAK

11:30 AM – 12:00 PM SESSION 11

Panel Discussion

SESSION CHAIR:

Elaine Morefield, PhD
Deputy Office Director, Office of New Drug Quality Assessment
CDER, FDA, US

This session will provide participants with a last opportunity for questions as a wrap-up of the 2.5-day workshop.

Jean-Louis Robert, PhD
Head, Medicines Control Laboratory, National Health Laboratory, Luxembourg

Diana Amador Toro, PhD
Director, New Jersey District, Office of Regulatory Affairs
FDA, US

Ana Carolina Araujo, PharmD
Sanitary Surveillance and Regulation Specialist – Pharmacist, Post-Approval Coordination Group (COPRE), Pharmaceutical Technology General Office (GTFAR)
ANVISA, Brazil

Christine Moore, PhD
Director, Office of New Drug Quality Assessment
CDER, FDA, US

12:00 PM WORKSHOP ADJOURNED