CMC Workshop 2015

April 13-15

Hyatt Regency Bethesda, Bethesda, MD

As of April 7, 2015

PROGRAM CHAIR:

Yasmin de Faria Krim, PharmD, MScRA
Senior Manager
Pharma Technical Regulatory
F. Hoffmann-La Roche Limited, Switzerland

PROGRAM COMMITTEE:

Christine Anderson, MS, RAC
Senior Research Scientist
Global Regulatory Affairs CMC
Eli Lilly and Company

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Vertex Pharmaceuticals

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Ramesh K. Sood, PhD
Senior Scientific Advisor (Acting)
Office of New Drug Products
CDER, FDA

Thirunellai G. Venkateshwaran, PhD
Senior Director
Pharma Technical Regulatory
Genentech, A Member of the Roche Group

OVERVIEW:

DIA, in cooperation with the American Association of Pharmaceutical Scientists (AAPS), will host a two and a half day workshop that will focus on the current Chemistry, Manufacturing, and Controls (CMC) challenges facing the global pharmaceutical and biopharmaceutical communities from development, implementation, and regulatory perspectives. This interactive workshop offers both plenary and parallel breakout sessions, featuring cross-functional discussions on numerous hot topics.

LEARNING OBJECTIVES:

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

- Discuss the current CMC topics for chemical and biological products in a globalized environment
- Explain current issues in the area of life cycle management
- State updates in the post-approval regulatory landscape in Latin America and Asia Pacific
- Explain the current situation in the area of QbD and its implementation
- Discuss control strategy, continuous manufacturing, and process validation
- Indicate recent initiatives for quality metrics, breakthrough therapies, and risk-based review
- Recognize issues in the development of pediatrics
- Describe the implementation of global standards for control of elemental impurities

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

aaps® American Association of Pharmaceutical Scientists

DIA DEVELOP INNOVATE ADVANCE

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Horsham, PA 19044 USA

This program is co-sponsored by the American Association of Pharmaceutical Scientists

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CONTINUING EDUCATION

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CONTINUING EDUCATION PHARMACY ALLOCATION

The following sessions are approved for ACPE credit:

- **Session 1: Life Cycle Management:** 1.5 contact hours or .15 CEUs; 0286-0000-15-048-L04-P
- **Session 2B: Post-Approval Change Management Protocols (PACMP):** 1.5 contact hours or .15 CEUs; 0286-0000-15-049-L04-P
- **Session 4: Control Strategy Approaches for Drug Substance and Drug Product:** 2 contact hours or .2 CEUs; 0286-0000-15-050-L04-P
- **Session 5B: Risk-based Review:** 1.5 contact hours or .15 CEUs; 0286-0000-15-051-L04-P
- **Session 6B: Pediatrics:** 2 contact hours or .2 CEUs; 0286-0000-15-055-L04-P
- **Session 6C: Comparability Biologicals:** 2 contact hours or .2 CEUs; 0286-0000-15-053-L04-P
- **Session 8C: Risk Management for Biologicals:** 2 contact hours or .2 CEUs; 0286-0000-15-054-L04-P
- **Session 9B: Breakthrough Therapies:** 1.5 contact hours or .15 CEUs; 0286-0000-15-052-L04-P
- **Session 9C: Clinically Relevant Specifications: Technical Considerations and Regulatory Expectations:** 1.5 contact hours or .15 CEUs; 0286-0000-15-056-L04-P

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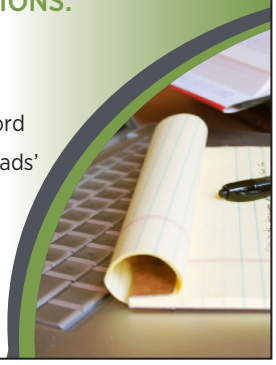

MONDAY, APRIL 13

7:30-8:30AM **REGISTRATION/CONTINENTAL BREAKFAST/EXHIBITS**

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

PROGRAM CHAIR:

Yasmin de Faria Krim, PharmD, MSc
Senior Manager
Pharma Technical Regulatory
Hoffmann-La Roche Limited, Switzerland

8:45–10:15AM SESSION 1: LIFE CYCLE MANAGEMENT

SESSION CHAIRS:

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

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

The concepts described in ICH Q8, Q9, Q10 and Q11 provide opportunities for a more science- and risk-based approach for developing medicinal products. However full implementation of these concepts have not been realized mainly with regards to assessing changes across the life cycle of pharmaceutical products. Several gaps exist which limit intended benefits: criteria for a harmonized risk-based change management system that effectively evaluates the impact of change on quality, clarity of the expectations of a knowledge management system that ensures continuity of product and process information, and appropriate level of detail and information sufficient for regulatory assessment and inspection.

Leading regulators from Europe and the United States will join industry speakers to share their perspectives on this topic and provide an update on progress made to date. Presentations will be followed by panel discussions.

Life Cycle Management – ICH Q12

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

Life Cycle Management - Industry Perspective and Case Studies

Ganapathy Mohan, PhD
Head Global CMC
Merck, Sharp and Dohme, Corp.

Life Cycle Management – EU Perspective

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

Life Cycle Management – FDA Perspective

Robert Iser, MS
Senior Scientific Advisor (Acting)
Office of Pharmaceutical Quality
CDER, FDA

10:15-10:45AM **REFRESHMENT BREAK/EXHIBITS**

10:45AM-12:15PM **SESSION 2**

TRACK A:

Technology Transfer

SESSION CHAIR:

C. Narahari Rao
Senior Scientist
Purification Process Development
Bristol-Myers Squibb

Analytical Method Transfers: Practice and Pitfalls

Wayland Rushing, PhD
Sr. Scientific Advisor
ABC Labs

Technology Transfer: CMC activities for the manufacture of monoclonal antibodies

Patricia M. Seymour Sr.
Consultant
BioProcess Technology Consultants, Inc.

TRACK B:

Post-approval Change Management Protocols (PACMP)

SESSION CHAIRS:

Nirdosh Jagota, PhD
Vice President Regulatory - Small Molecules
Genentech, A Member of the Roche Group, US

Wendy Mavroudakis
Senior Director
Global Regulatory Affairs
Johnson & Johnson Pharmaceutical R&D LLC

The focus of this session will be on post approval change management and its application for products in various stages of the life cycle. The speakers will touch on risk based approaches to manage post approval changes and various approaches used in both the generics as well as the branded pharma industry. The speakers will also discuss some of the challenges in the global implementation of changes and discuss steps that need to be taken to facilitate a consistent post approval management pathway that will enable innovation in the industry and help in the utilization of lean principles within the industry.

Post Approval Change Management: FDA Perspective

Christine M. V. Moore, PhD
Acting Director
Office of Process and Facilities
CDER, FDA

Post Approval Life Cycle Change Management - Completing What Quality by Design Intended to Deliver

Roger Nosal
Vice President and Head
Global CMC
Pfizer Inc.

GDUFA Impacts on Post-Approval Changes – The Real Deal

Barinder Sandhu, MBA, RAC
Senior Director
Regulatory Affairs, US Generics
Teva Pharmaceuticals

TRACK C:

Case Studies and Post Approval Change Requirements in Latin America

SESSION CHAIR:

Bekki Thomas, MS

Multi-national companies are challenged with the rapidly evolving regulatory environment in Latin America. The region has diverse country specific requirements that impact NCE's and life cycle management of the assets. An industry speaker will present an overview of post approval changes and the impact on industry followed by an update on challenges with international harmonization and RDC 58 discussion presented by the Office of Evaluation of Post Approval Changes of the Brazilian Health and Surveillance Agency. Example case studies of simple post approval changes and the impact to industry will be presented. The session will include an opportunity for CMC experts to ask questions and discuss opportunities for convergence or regionalization of the regulatory requirements.

Latin America - Post Approval Changes and the Industry Perspective

Ivone Takenaka, PhD
Associate Director
Global Regulatory - CMC
Bristol-Myers Squibb Co.

Post-approval Changes in Brazil: Current Scenario and Perspectives

Raphael Sanches Pereira
Health Regulation Expert
Office of Evaluation of Post Approval Changes of Synthetic Drugs/GEPRE/SUMED
Anvisa, Brazilian Health and Surveillance Agency, Brazil

Post Approval Change Requirements and Discussion of New Quality Consultation Guidance

Marcio Silva
Regulatory Governance / P&I Manager
Emerging Markets & Asia Pacific – Brazil
GlaxoSmithKline, Brazil

12:15-1:30PM LUNCH/EXHIBITS

1:30-3:00PM **SESSION 3**

TRACK A:

Elemental Impurities

SESSION CHAIR:

John F. Kauffman, PhD
Deputy Director
Division of Pharmaceutical Analysis
CDER, FDA

This session will address implementation of recently developed standards and guidelines for control of elemental impurities in drug products. Topics will include implementation of ICH Q3D: Guideline on Elemental Impurities and an update on USP chapters <232> and <233>. An industry perspective on the standards and guidelines will also be presented. At the conclusion of the presentations, the panel of speakers will be available to answer questions from the audience.

Elemental Impurities: FDA Perspective

Danae Christodoulou, PhD
Acting Branch Chief
Office of Pharmaceutical Quality, ONDP
CDER, FDA

USP Elemental Impurities: An Update

Kahkashan Zaidi, PhD
Principal Scientific Liaison
USP

Industry Perspective on Elemental Impurity Standards and Guidelines

Melissa Figgins
Director PMO
Sandoz

TRACK B:

Quality Metrics

SESSION CHAIR:

Peter Carbone
Novartis Pharmaceutical

OPQ's role in CDER's 21st Century Quality Initiative

Ashley Boam
Acting Director
Office of Policy for Pharmaceutical Quality
CDER, FDA

Industry Perspective

Laura Cannon
Senior Director Quality
TEVA

TRACK C:

Post-Approval Regulatory Landscape in Asia Pacific – Challenges to Multi-National Companies

SESSION CHAIR:

Chi-wan Chen, PhD
Executive Director
Global CMC
Pfizer Inc.

In the rapidly growing emerging market, multi-national companies (MNCs) face tremendous challenges in ensuring that they are in compliance with regional and country-specific regulations and policies while maintaining the drug supply to patients throughout the product life cycle. Nowhere are these challenges as pronounced as the Asia Pacific region whose 15 countries/autonomies have extremely diverse regulatory histories, systems, and capacities. This session will provide a close examination of the post approval landscape across the region and an opportunity for MNC regulatory CMC experts to share their experiences with the audience.

What is New in China?

Chi-wan Chen, PhD
Executive Director
Global CMC
Pfizer Inc.

ASEAN Region – Is it Harmonized?

Susan E. Stolz
Sr. Research Scientist
CMC Regulatory Affairs
Eli Lilly and Company

Experiences in Other Asian Countries – Japan, South Korea, and Taiwan

Nirdosh Jagota, PhD
Vice President Regulatory - Small Molecules
Genentech, A Member of the Roche Group, US

3:00-3:30PM **REFRESHMENT BREAK/EXHIBITS**

3:30–5:30PM **SESSION 4**

Control Strategy Approaches for Drug Substance and Drug Product

SESSION CHAIR:

Elaine Morefield, PhD, RPh
Senior Director
CMC Strategy, R&D
Vertex Pharmaceuticals

This session will look at approaches to developing, maintaining, and improving the control strategy throughout the product life cycle, from a drug substance, drug product, and regulatory perspective. Following the presentations, a discussion of control strategy approaches in described situations will allow the audience to share their insights and approaches and to ask questions of the panel members.

Life Cycle Approach to Drug Substance Development

Nick Thomson, PhD
Director Technology API
Chemical Research and Development
Pfizer Ltd.

Development and Continual Improvement of the Drug Product Control Strategy

Stephen Tyler
Director
Quality Assurance
AbbVie

Regulatory Considerations for the Development of Control Strategy

Sarah Pope Miksinski, PhD
Acting Director
Office of Pharmaceutical Quality, Office of New Drug Products
CDER, FDA

TUESDAY, APRIL 14

7:00-8:00AM REGISTRATION/CONTINENTAL BREAKFAST/EXHIBITS

8:00–9:30AM SESSION 5

TRACK A:

Continuous Manufacturing

SESSION CHAIRS

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

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

Continuous manufacturing is gaining momentum in industry and is supported by major regulators. This session will assess industry implementation strategy, the current regulatory environment, and existing guidelines and their impact on manufacturing innovation. Presentations will outline key industry and regulatory concerns, gaps, challenges, and propose a way forward to facilitate speedy implementation.

Industry and regulatory presentations will be followed by panel discussion.

Regulatory and Quality Considerations for Continuous Manufacturing

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

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

Continuous Manufacturing – Implementation Strategy: Industry Perspective

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

Continuous Manufacturing – Regulatory Challenges and Opportunities

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

TRACK B:

Risk-based Review

SESSION CHAIR:

Nirdosh Jagota, PhD
Vice President Regulatory - Small Molecules
Genentech, A Member of the Roche Group

Patient confidence in the quality of a product relies on regulatory approval of a product registration, which in turn relies on a company's effective demonstration and conveyance of control of the quality attributes of that product. Benefits of a product with respect to patient needs have to be appropriately balanced with management of risks and uncertainty. This session will focus on how quality attributes can be conveyed in a product registration to effectively demonstrate how quality attributes are controlled and managed and can be reviewed to provide confidence in the quality of a product.

Confidence in Quality: A Proposal to Unify Approaches to Risk-based Regulatory Review

Roger Nosal
Vice President and Head
Global CMC
Pfizer Inc.

Presentation Title TBD

Diane J. Zezza, PhD
Vice President
Global Regulatory Affairs, CMC
Novartis Pharmaceuticals Corporation

Integrated Quality Assessment: An Update on Team-Based Review and One Quality Voice

Sarah Pope Miksinski, PhD
Acting Director
Office of Pharmaceutical Quality
Office of New Drug Products
CDER, FDA

TRACK C:

Process Validation/Verification – Biologicals

SESSION CHAIR:

Kowid Ho, PhD
Pharma Technical Regulatory Policy
F. Hoffmann-La Roche Ltd.

The product life cycle concept was introduced in ICH Q8 and Q10 guidelines. ICH Q11 describes process validation in a life cycle context that can include the collection and evaluation of data, from the process design stage throughout production.

These concepts are being implemented in ICH regions. This session will discuss the regulatory expectations and implementation of these concepts in the US and EU.

Integration of Control Strategy, Process Validation and Life cycle Management for Biologics Process Validation

Ronald Bates
Bristol-Myers Squibb

Implementation of Evolving Process Validation Principles for Legacy Products

Marco Strohmeier, PhD
Manager
Quality Assurance Improvement & Reliability
Roche Diagnostics GmbH, Germany

9:30-10:00AM REFRESHMENT BREAK/EXHIBITS

10:00AM–12:00PM SESSION 6

TRACK A:

Process Validation/Verification – Chemicals

SESSION CHAIR:

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

With the changing landscape of small molecule syntheses, and the implementation of many Quality by Design principles into the drug development as well as commercialization activities, traditional approaches to process validation may not provide a sufficient data package or confidence to ensure robust long term manufacture. Infrequent production, desired flexibility, and alternative synthetic platforms (i.e. flow chemistry) offer unique challenges and therefore validation or ongoing verification strategies that support these activities need to be considered. This session will provide examples of validation and verification strategies useful for addressing the changing regulatory landscape. Time will be reserved for a panel discussion and audience participation is highly encouraged.

Validation Challenges of a Small Molecule Flow Chemistry Process

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

FDA Perspective

Christina Capacci-Daniel, PhD
Consumer Safety Officer
Office of Process and Facilities
Office of Pharmaceutical Quality
CDER, FDA

TRACK B:

Pediatrics

SESSION CHAIR:

Peter Richardson, PhD
Head of Quality
Human Medicines Evaluation Division
European Medicines Agency, UK

This session will look at some of the issues arising during development of pediatric formulations; covering industry experiences and reflecting on the regulatory requirements in both the USA and EU. Progress continues to be made in this area and EMA has recently reviewed guidance at a workshop with industry. Outcomes from the workshop and how these may facilitate further evolution of this topic will be considered by speakers from both industry and regulatory authorities.

Challenges Associated with Developing Medicines for Children: An Industry Perspective

Gossett Campbell, PhD
Senior Scientific Investigator
Global Formulation Development
GlaxoSmithKline

Navigating the Pediatric Formulation Landscape: "Considerations for a Successful Global Journey"

Robert L. Ternik, PhD
Senior Research Advisor
Eli Lilly and Company

Pediatric Medicines – EU Regulatory Perspective

Piotr Kozarewicz, MSc
Head of Evaluation Procedures Service B
European Medicines Agency, UK

This presentation will be given via WebEx

TRACK C:

Comparability Biologicals

SESSION CHAIR:

Anthony Ridgway, PhD
Senior Regulatory Scientist
Office of the Director
Health Canada, Canada

An Industrial View of Comparability Applied to Biopharmaceuticals

Anthony S. Lubiniecki, ScD
Senior Scientific Director & Fellow
Janssen R&D LLC

FDA Perspective

Linan Ha, PhD
Team Leader
Division of Biotechnology Review & Research I
Office of Biotechnology Products, Office of Pharmaceutical Quality
CDER, FDA

European Perspective

Kowid Ho, PhD
Pharma Technical Regulatory Policy
F. Hoffman-La Roche, Ltd.

12:00-1:30PM **LUNCH/EXHIBITS**

1:30–3:00PM **SESSION 7**

Implementation of QbD

SESSION CHAIR:

Peter Richardson, PhD
Head of Quality
Human Medicines Evaluation Division
European Medicines Agency, UK

Industry and regulators have invested substantial resources in the Quality by Design project over recent years. This session will look at some of the gains that have been made for both small (chemical) and large (biological) molecules in this area and address real benefits versus expectations. The field has matured significantly with the implementation of ICH Q8, 9, 10, and 11 guidelines; however, there still remain opportunities to facilitate implementation. Recent progress in the area will be reviewed, such as outcomes from EMA QbD workshop and other FDA initiatives, with perspectives from both industry and regulators.

The Present and Future of QbD for New Drugs: An FDA Perspective

Sharmista Chatterjee, PhD
CMC Lead for QbD
Office of New Drug Quality Assessment
CDER, FDA

QbD Where Next – Industry Experience and Perspectives

Frank Montgomery
Global Head Regulatory CMC
AstraZeneca, UK

Targeting "Quick but Decisive" Analytics to Support Advanced Process Controls

Kazumi Kobayashi, PhD
Senior Principal Scientist
Technical Development
Biogen Idec

3:00-3:30PM **REFRESHMENT BREAK/EXHIBITS**

3:30–5:30PM **SESSION 8**

TRACK A:

Models, Control Strategy and Regulatory Implications: Some Thoughts and Examples from Industry

SESSION CHAIR:

Frank Montgomery
Global Head Regulatory CMC
AstraZeneca, UK

The Points to Consider ICH Endorsed Guide for ICH Q8/Q9/Q10 Implementation describes the use of models and their categorization from high to low impact depending on level of risk to patient when used as part of the control strategy. There are a variety of ways that this information can be incorporated into the control strategy to assure product quality and process consistency as part of a marketing application. This session will look at a variety of ways that models have been used to support marketing applications, the data and statistical analysis that was needed gain approval, and some considerations of how this could evolve in the future. For example a Design Space may be associated with RTRT, or may be part of a control strategy that includes extensive end-product testing; consequently the degree of assurance and the level of statistical confidence required can vary.

Model Implementation: It Really Is All About the Control Strategy

John Lepore, PhD
QbD and CMC Practices Lead
Global Pharmaceutical Commercialization
Merck

Science and Strategy: The Role of Models in Achieving Manufacturing Flexibility

Matt Popkin, PhD
Product Development Global Regulatory Liaison
GlaxoSmithKline, UK

Statistical Models and Tools for the Assessment of Process Parameter Criticality

Nathan Ide, PhD
Senior Principal Scientist
Pfizer

TRACK B:

FDA/EMA QbD Pilot Program Update

SESSION CHAIR:

Ganapathy Mohan, PhD
Head of Global CMC
Merck & Co., Inc.

Quality by Design (QbD) has become a way of life in the pharmaceutical industry in how we develop drugs. This was piloted in the early 2000s (around 2004-2005) by the US FDA and submissions were filed by sponsors for review and approval by the US FDA. In the years since the pilot program was started, a number of continuous improvements have been made to the understanding, expectations of regulators, and the implementation from the industry. However, the expectations from the regulators around the world have not been fully harmonized. In 2011, FDA and EMA announced the joint QbD Pilot Program which would help to bring the review process closer than before between the two regulatory bodies.

This session will have leading regulators, from Europe and the United States, and an industry speaker share their perspectives on this topic and provide an update on progress made to date. Presentations will be followed by panel discussions.

US FDA Regulatory Perspectives of the FDA/EMA QbD Pilot Program

Christine M. V. Moore, PhD
Acting Director
Office of Process and Facilities
CDER, FDA

Industry Perspectives of the FDA/EMA QbD Pilot Program

Roger Nosal
Vice President and Head
Global CMC
Pfizer Inc.

EU Regulatory Perspectives of the FDA/EMA QbD Pilot Program

Jean-Louis Robert, PhD
Head
Pharmaceutical Chemistry Unit
National Health Laboratory, Luxembourg

TRACK C:

Risk Management for Biologicals

SESSION CHAIR:

Peter Richardson, PhD
Head of Quality
Human Medicines Evaluation Division
European Medicines Agency, UK

This session will look at some of the issues arising in managing risks for large (biological) molecules, mainly from the perspective of QbD. ICH guidelines detail approaches to risk management, however interpretation of these concepts have not always been consistently applied to the development of control strategies for biological substances. Of particular importance is providing assurance that Critical Quality Attributes have been appropriately identified and placed in the control strategy. Risk ranking and filtering is an important tool for this purpose, yet other tools can be employed to achieve this. Speakers with industry and regulatory experience will share their perspectives on how this may be applied for innovator and biosimilar products.

Fit For Purpose Quality Attribute Risk Assessments

Patrick Swann, PhD
Senior Director
Technical Development
Biogen Idec

Risk Management Applied to Control Strategy

Kowid Ho, PhD
Pharma Technical Regulatory Policy
F. Hoffmann-La Roche Ltd.

Quality Risk Management in the Biosimilar Development

Martin Schiestl, PhD
Scientific & Regulatory Advisor
Sandoz GmbH, Austria

WEDNESDAY, APRIL 15

7:00-8:00AM **REGISTRATION/CONTINENTAL BREAKFAST/EXHIBITS**

8:00–9:30AM **SESSION 9**

TRACK A:

API Starting Materials – Implementation

SESSION CHAIR:

D. Scott Coffey, PhD
Sr. Director CMC, Chorus and Global External R&D
Eli Lilly and Company

API starting material selection and justification is a critical activity in the drug development and commercialization process. General principles for the selection of API starting materials are outlined in ICH Q11. The application of these guidelines, however, has varied from company to company, and interpretation of the guidelines by pharmaceutical companies and regulatory agencies has been inconsistent. This has led to frustration by both industry and regulatory agencies and can lead to delays in drug development timelines and to significant changes in supply chain strategies. This session will provide examples of the implementation of API starting material strategies, feedback from regulatory agencies, and a regulatory agency view on API starting materials. Time will be reserved for a panel discussion, and audience participation is highly encouraged.

Definition of Starting Materials for the Synthesis of the Active Substance – The EMA View

Robert Bream, PhD
Quality Specialist
European Medicines Agency, UK

This presentation will be given via WebEx

Science and the Supply Chain: Challenges in the Application of ICHQ11 Principles for the Selection and Justification of API Starting Materials

Michael A. McGuire, PhD
Quality by Design Lead/ CMC Technical Lead
GlaxoSmithKline

Recent Approaches to Registered Starting Materials

John Pavey, PhD
Director
Global Chemical Development
AstraZeneca, UK

TRACK B:

Breakthrough Therapies

SESSION CHAIR:

Ramesh K. Sood, PhD
Senior Scientific Advisor (Acting)
Office of New Drug Products
CDER, FDA

Section 902 of the Food and Drug Administration Safety and Innovation Act (FDASIA), passed in year 2012, provides a roadmap for expedited development and review of drugs for serious or life-threatening diseases or conditions where the preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. This has provided an excellent opportunity for the pharmaceutical industry and the regulators to develop and make available medically necessary treatments to the American public. The development and approval of such therapies typically involve a rapid manufacturing development program to accommodate the accelerated pace of the clinical development.

Regulators from the Food and Drug Administration and pharmaceutical industry speakers will share their experiences, successes, and challenges faced during the development and approval process of such therapies. Presentations will be followed by panel discussions.

Breakthrough Therapy Drugs: An Industry Perspective

Brian Kelley, PhD
Vice President
Bioprocess Development
Genentech, A Member of the Roche Group

An Industry Experience With Breakthrough Therapy Drugs

John Groskoph, MBA
Senior Director
Pfizer Inc.

CMC Submission Strategies for Breakthrough Therapies: Successes and Challenges - FDA Perspective

Dorota Matecka, PhD
Branch Chief (Acting)
Office of New Drug Products
CDER, FDA

Manufacturing Challenges for Breakthrough Therapies

Robert Wittorf, PharmD
Pharmacist
Office of Pharmaceutical Quality
Office of Process and Facilities
CDER, FDA

TRACK C:

Clinically Relevant Specifications: Technical Considerations and Regulatory Expectations

SESSION CHAIR:

Nagesh Bandi, PhD
Director
Global CMC
Pfizer Pharmaceuticals, Inc.

Due to the critical role that dissolution plays in the bioavailability of a drug, in vitro dissolution can serve as a relevant predictor of the in vivo performance of the drug product. There is growing evidence that clinically meaningful dissolution specifications will minimize variability to the patient and therefore will optimize drug therapy. This session will discuss the relevant CMC and clinical pharmacology-related factors that should be considered in developing a clinically relevant dissolution method and specifications. In addition, the regulatory expectations and considerations will be illustrated.

Novel Approaches to Better Understand Drug Product Design and Testing

Phillip Floyd, PhD
Head
Analytical Development
GlaxoSmithKline

Strategies for Ensuring Dissolution and Clinical Performance

Vivek Purohit, PhD
Director
Pfizer Inc.

Clinically Relevant Specifications: FDA Perspective

Angelica Dorantes, PhD
Biopharm Lead
Office of Pharmaceutical Quality
CDER, FDA

9:30-10:00AM **REFRESHMENT BREAK/EXHIBITS**

10:00AM–12:00PM **SESSION 10**

Updates from Regulatory Agencies (Dossier Review/Inspections) FDA, EMA and Other Global Agencies

SESSION CHAIR:

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

To conclude the workshop, this last session will provide updates from experts from regulatory agencies as well as a true opportunity for a dialogue with the audience.

SPEAKER EU:

Peter Richardson, PhD
Head of Quality
Human Medicines Evaluation Division
European Medicines Agency, UK

SPEAKER BRAZIL:

Raphael Sanches Pereira
Health Regulation Expert
Office of Evaluation of Post Approval Changes of Synthetic Drugs/GEPRE/SUMED
Anvisa, Brazilian Health and Surveillance Agency, Brazil

SPEAKER JAPAN:

Yoshihiro Matsuda
Deputy Director
Office of Standards and Guidelines Development
Pharmaceuticals and Medical Devices Agency (PMDA), Japan

SPEAKER FDA:

Lawrence Yu, PhD
Deputy Director, Office of Pharmaceutical Quality
CDER, FDA, US

Panel Discussion

ALL PARTICIPANTS ABOVE AND:

Anthony Ridgway, PhD
Senior Regulatory Scientist
Office of the Director
Health Canada, Canada

12:00PM **WORKSHOP ADJOURNED**