

CDER SMALL BUSINESS AND INDUSTRY ASSISTANCE (SBIA)

REGULATORY EDUCATION FOR INDUSTRY (REdI)

Annual Conference 2023



JUNE 5-9

VIA WEBCAST | www.fda.gov/CDERSBIA

Version 7 – Updated June 4, 2023

For files and resources, please visit
[The Event Page on SBIAevents.com](https://www.fda.gov/sbiaevents)

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AGENDA

All times are Eastern (EDT UTC-4)

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June 5-6

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June 8-9

DAY ONE: Keynote & Plenary: Monday, June 5, 2023

8:40– 9:00

SBIA 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

9:00 – 9:15

FDA Welcome

Robert M. Califf, MD

Commissioner of Food and Drugs

Food and Drug Administration

9:15 – 10:45

Plenary: User Fee Impact on FDA Programs

Moderated by:

Elias Mallis

Director, Division of Industry and Consumer Education (DICE)

Office of Communication and Education (OCE)

Center for Devices and Radiological Health (CDRH)

Jeff Shuren, MD, JD

Director

Center for Devices and Radiological Health (CDRH)

Patrizia Cavazzoni, MD

Director

Center for Drug Evaluation and Research (CDER)

Peter Marks, MD, PhD

Director

Center for Biologics Evaluation and Research (CBER)

10:45 - 11:00 AM: BREAK

DAY ONE: CDER Sessions: Monday, June 5, 2023

Your CDER Hosts		
Renu Lal, PharmD <i>Lieutenant Commander, USPHS</i> DDI OCOMM CDER	Nora Lim, PharmD, BCPS <i>Lieutenant, USPHS, Pharmacist</i> SBIA DDI OCOMM CDER	Forest "Ray" Ford, PharmD, BCPS <i>Captain, USPHS</i> DDI OCOMM CDER

11:00 - 11:30

Biosimilar Program Updates and What's New Under BsUFA III

This presentation will review the new BsUFA III commitments, focusing on supplement categories and associated timelines, guidance commitments and regulatory science.

The presentation will provide an overview of the new BsUFA regulatory science program commitments, research priorities, goals, and objectives. In addition, the speakers will describe and explain research that can improve the efficiency of biosimilar development and advance the development of interchangeable products and the regulatory impact.

Legislative updates will also be reviewed that impact biosimilar development and 351(a) and 351 (K) BLA license holders. This presentation will also describe and explain the resources available for health care providers and other stakeholders to learn more about biosimilar and interchangeable biosimilars through the Purple Book and other FDA educational resources.

Stacey Ricci, M.Eng, ScD,
Director of Scientific Review Staff (SRS)
Office of Therapeutic Biologics and Biosimilars (OTBB)
Office of New Drugs (OND) | CDER

Kimberly Maxfield, PhD
BsUFA Regulatory Science Program Coordinator
OTBB | OND | CDER

11:30 – 12:00

FDA Formal Meetings: What's New Under PDUFA, BsUFA, and OMuFA

This session will provide an overview of FDA formal meetings under reauthorizations of PDUFA and BsUFA as well as the first authorization of OMuFA. In addition, updates to FDA's transition to in-person face-to-face meetings will be provided.

Elizabeth Thompson, MS
Chief Project Management Staff
Division of Regulatory Operations for Nonprescription Drugs (DRO-NPD)
Office of Regulatory Operations (ORO)
OND | CDER

12:00 – 12:15

Question and Answer Panel

Stacey Ricci, Kimberly Maxfield, Elizabeth Thompson

12:15 - 12:45 PM: LUNCH BREAK

12:45 – 12:50

Welcome Back

DAY ONE: CDER Sessions: Monday, June 5, 2023

12:50 – 1:20

ESG (Electronic Submissions Gateway)...The Road to Modernization

This presentation will outline the evolution and modernization of ESG along with plans for the next generation.

Jessica Bernhardt, MS

AdminApps Program Manager, ESG Program Manager
Office of Information Management & Technology (OIMT)
Office of Digital Transformation (ODT)
Office of the Commissioner (OC)

1:20 – 1:40

eCTD v4.0 and Latest on eCTD

This session will provide an FDA eCTD v4.0 Implementation Update.

Jonathan Resnick

Project Management Officer
Division of Data Management Services and Solutions
(DDMSS)
Office of Business Informatics (OBI)
Office of Strategic Programs (OSP) | CDER

1:40 – 2:00

Electronic Submission Practicalities and Application Tips

Metrics, best practices, and most common validation errors.

Heather Crandall

Operations Research Analyst
DDMSS | OBI | OSP | CDER

2:00 – 2:15

Question and Answer Panel

**Jessica Bernhardt, Jonathan Resnick,
Heather Crandall**

2:15 – 2:30 PM: BREAK

DAY ONE: CDER Sessions: Monday, June 5, 2023

2:30 – 3:00

Data Standards

This presentation will be an overview of the CDER-CBER Data Standards Program (DSP). It will also highlight a number of the OSP's key Initiatives along with updates on current project progress.

Hao (Ray) Wang
Director
 Data Standards Staff (DSS)
 Office of Strategic Program (OSP) | CDER

3:00 – 3:30

PDUFA VII Goals for Digital Health Technologies - A Regulatory Review Perspective

Section IV.C of the PDUFA VII commitment letter describes FDA's goals to enhance the use of DHT-generated data in drug development and review. This presentation takes a look at this data and its use from a regulatory review perspective.

Andrew Potter
Mathematical Statistician
 Division of Biometrics I (DBI)
 Office of Biostatistics (OB)
 Office of Translational Sciences (OTS) | CDER

3:30 – 4:00

PDUFA VII Goals For Digital Health Technologies (DHT) - An IT Perspective

Section IV.C of the PDUFA VII commitment letter describes FDA's goals to enhance the use of DHT-generated data in drug development and review. This presentation expands on the previous presentation to discuss activities underway and planned to support submission and review of DHT-generated data.

Mary Ann Slack
Director
 OSP | CDER

4:00 – 4:15

Question and Answer Panel

**Hao (Ray) Wang, Andrew Potter, and
 Mary Ann Slack**

4:15 – 4:25

Day One Closing

Forest "Ray" Ford, PharmD, BCPS
Captain, United States Public Health Service
 DDI | OCOMM | CDER

4:25 PM: DAY ONE ADJOURN

DAY TWO: CDER Sessions: Tuesday, June 6, 2023

8:30 – 8:45

Day Two Welcome and Overview

Renu Lal, PharmD
Lieutenant Commander, USPHS
 DDI | OCOMM | CDER

Nora Lim, PharmD, BCPS
Lieutenant, USPHS, Pharmacist
 SBIA | DDI | OCOMM | CDER

Forest "Ray" Ford, PharmD, BCPS
Captain, USPHS
 DDI | OCOMM | CDER

8:45 – 9:15

Leveraging SBIA's Resources

Includes Question and Answer Session

Renu Lal, PharmD, BCACP
Lieutenant Commander
 United States Public Health Service (USPHS)
 Team Lead, Division of Drug Information (DDI)
 Deputy Director, SBIA
 OCOMM | CDER

9:15 - 9:45

Overview of FDA Split Real Time Application Review (STAR) Pilot Program

The FDA will present an overview of the CDER STAR Pilot Program and discuss considerations and criteria for applications that may qualify for the program.

LaShawn Schnupp, PharmD
Senior Regulatory Health Project Manager
 STAR Program Manager
 Program Development, Implementation,
 and Management Staff (PDIMS)
 Office of Program Operations (OPO) | OND | CDER

J. Paul Phillips, MS
Director
 OPO | OND | CDER

9:45 – 10:30

Use-Related Risk Analysis (URRA) and Human Factor (HF) Protocol Reviews: What to Submit for an Efficient Review

The FDA will discuss:

- The HF commitments under PDUFA VII and BSUFA III
- Tips for efficient review of HF Protocol
- Tips for efficient review of URRA

Lolita Sterrett, PharmD
Associate Director for Human Factors
 Division of Medication Error Prevention and
 Analysis 2 (DMEPA 2)
 Office of Medication Error Prevention and Risk
 Management (OMEPRM)
 Office of Surveillance and Epidemiology (OSE)
 CDER

10:30 – 10:45

Question and Answer Panel

**LaShawn Schnupp, J. Paul Phillips,
 and Lolita Sterrett**

10:45 – 11:00: BREAK

DAY TWO: CDER Sessions: Tuesday, June 6, 2023

11:00 – 11:30

The Modernization of Clinical Trials through Digital Health Technologies (DHT), Decentralized Clinical Trials (DCT) and Point of Care Trials

The FDA will discuss how clinical trials are advancing through the use of DHTs, DCTs and point of care trials. FDA will provide an overview of DHT PDUFA VII commitments.

Beth Kunkoski
Health Science Policy Analyst
 Clinical Methodologies
 Office of Medical Policy (OMP) | CDER

11:30 – 12:00

PDUFA VII Real-World Evidence

The FDA will discuss the new provisions under PDUFA VII: Advancing Real-World Evidence (RWE) for Use in Regulatory Decision Making

Kimberly Smith
CAPT, USPHS
 Real-World Evidence (RWE) Analytics
 OMP | CDER

12:00 - 12:15

Question and Answer Panel

Beth Kunkoski and Kimberly Smith

12:15 – 12:45: LUNCH BREAK

12:45 – 12:50

Welcome Back

12:50 – 1:20

PDUFA VII PMR (Postmarketing Requirements) Commitments: Preapproval & Postapproval

The FDA will discuss the new PMR commitments under PDUFA VII:

- The timing for communicating "anticipated" PMRs during the preapproval process
- The postapproval process for responding to PMR release requests

Kathleen (Kathy) Weil
Senior Science Policy Analyst
 PMR/PMC Program Manager
 Safety Policy Research and Initiatives Team (SPIRIT)
 Immediate Office | OND | CDER

1:20 – 1:50

How CDER is Accelerating Rare Disease Cures and the PDUFA VII Rare Disease Endpoint Advancement Pilot Program

This session will provide an overview on how CDER is working to advance rare disease cures with the Accelerating Rare disease Cures (ARC) Program, as well as updates on the PDUFA VII: Rare Disease Endpoint Advancement Pilot Program.

Kerry Jo Lee, MD
Associate Director for Rare Diseases
 Rare Diseases Team
 Division of Rare Diseases and Medical Genetics (DRDMG)
 Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORDPURM)
 OND | CDER

DAY TWO: CDER Sessions: Tuesday, June 6, 2023

1:50 – 2:20

PDUFA VII Chemistry, Manufacturing, and Controls (CMC) Assessment Updates

FDA will discuss quality assessment for products in expedited programs including an update on the Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot Program. FDA will also provide an update on new assessment tools and platforms including Knowledge-aided assessment & structured application (KASA).

Paresma Patel, PhD,
Director, Division of New Drug API (DNDAPL)
 Office of New Drug Products (ONDP)
 Office of Pharmaceutical Quality (OPQ) | CDER

2:20 – 2:35

Question and Answer Panel

Kathleen (Kathy) Weil, Kerry Jo Lee
 and **Paresma Patel**

2:35 – 2:50: BREAK

2:50 – 3:20

Best Practices for Human Drug Product Recalls

FDA will discuss considerations and best practices throughout a human drug recall life cycle including when to conduct a recall, reporting to FDA, implementing a recall, and evaluating recall effectiveness.

Doris Chin
Consumer Safety Officer
 Incidents, Recalls and Shortages Branch
 Division of Supply Chain Integrity
 Office of Drug Security, Integrity, and Response
 (OSDIR)
 Office of Compliance (OC) | CDER

3:20 – 3:50

A Rough Guide to Biologics Manufacturing

This presentation will include a basic background on how biological products such as monoclonal antibodies and other therapeutic proteins are regulated by FDA/CDER. It will discuss the unique factors for chemistry, manufacturing, and controls for biological products, including both scientific and regulatory nuances. The presentation will also address how CDER approaches inspectional activities for these biological products. Finally, it will identify common themes for that lead to complete responses for marketing applications for these products.

Joel Welch, PhD
Associate Director for Science & Biosimilar Strategy
 Chair for Emerging Technology Team
 Office of Biotechnology Products (OBP)
 OPQ | CDER

Chris Downey, PhD
Director, Division of Biotech Manufacturing
 Office of Pharmaceutical Manufacturing Assessment
 (OPMA) | OPQ | CDER

DAY TWO: CDER Sessions: Tuesday, June 6, 2023

3:50 – 4:05

Question and Answer Panel

Doris Chin, Joel Welch, Chris Downey

4:05 – 4:15

Day Two Closing

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

4:15 PM: DAY TWO ADJOURN

DAY THREE: CDRH Sessions: Wednesday, June 7, 2023

AGENDA

All times are Eastern (EDT UTC-4)

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June 5-6

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June 7-8

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June 8-9

8:30 – 8:45

Welcome to REdI 2023 Device Track, Part 1

Elias Mallis

Director

Division of Industry and Consumer Education (DICE)
Office of Communication and Education (OCE)
FDA Center for Devices and Radiological Health (CDRH)

8:45 – 9:25

Medical Device Regulatory Framework: Where to Start?

The Center for Devices and Radiological Health (CDRH) provides resources to help the medical device industry understand the regulatory requirements and process for marketing medical devices in the United States. This presentation will introduce the basics of medical device regulation, highlighting these helpful resources and empowering the medical device industry to find and use these resources. The topics we will discuss include: the definition of a medical device, general and special controls defined in regulation, the use of databases to seek information to support the regulatory processes, and a brief introduction of the different types of premarket pathways as they relate to regulatory requirements.

Kendra Holter, MSN, RN

Consumer Safety Officer

Premarket Programs Branch

DICE | OCE | CDRH

Suggested pre-requisites:

- [How to Study and Market Your Device](#)
- [Is My Product a Medical Device?](#)
- [How is My Medical Device Classified?](#)

9:25 – 10:05

Biocompatibility Basics

This session will provide an overview of the FDA Biocompatibility, including some key definitions. Participants will learn when and how biocompatibility is considered, and how to apply a risk-based approach for biocompatibility.

Jennifer Goode

Biocompatibility Program Advisor

Office of Product Evaluation and Quality (OPEQ)

CDRH

Suggested pre-requisites:

- [Use of International Standard ISO 10993-1, Biological evaluation of medical devices - Part 1](#)
- [Color Additives for Medical Devices](#)
- [Use of International Standard ISO 10993-1, "Biological evaluation of medical devices - Part 1](#)

10:05 - 10:25: BREAK

DAY THREE: CDRH Sessions: Wednesday, June 7, 2023

10:25 - 11:05

Appropriate Use of Voluntary Consensus Standards and the Conformity Assessment Program

A reliance upon consensus standards reduces regulatory burden, streamlines conformity assessment, enhances device quality and promotes global harmonization. When manufacturers cite FDA-recognized standards, uncertainty about conformity assessment documentation is reduced and less paperwork is required. This session introduces the audience to the practical aspects of citing standards in device submissions and offers tips on why and how to participate in the Accreditation Scheme for Conformity Assessment (ASCA), CDRH's new initiative to streamline conformity assessment in device review.

Scott A. Colburn*Director*Standards and Conformity Assessment Program
Office of Strategic Partnerships and Technology
Innovation (OST) | CDRH**Suggested pre-requisites:**

- [Standards and Conformity Assessment Program](#)
- [Module 1: Standards Overview](#)
- [Module 2: Standards: Resources and Use in Premarket Submissions](#)
- [Module 3: CDRH Standards Recognition Process](#)
- [Module 4: How to Use Consensus Standards in Premarket Submissions](#)
- [Module 5: The ASCA Pilot: Streamlining Conformity Assessment in Device Submission](#)

11:05 - 11:45

Detangling the 510(k) Process

The 510(k) process can be complicated and confusing. It can feel like you are tangled up in unknown requirements when all you want to do is help patients with your medical device. You may be asking yourself: What is substantial equivalence? Why do I need a predicate device and how do I find one? When will the review process be completed and what are the steps involved? The answers to these questions as well as helpful hints and tips will be explored in Detangling the 510(k) Process.

Andrew Sprau*Consumer Safety Officer*
Premarket Programs Branch
DICE | OCE | CDRH**Suggested pre-requisites:**

- [Premarket Notification 510\(k\)](#)
- [The 510\(k\) Program](#)

11:45 - 12:45: LUNCH BREAK

12:45 – 12:50

Welcome Back

DAY THREE: CDRH Sessions: Wednesday, June 7, 2023

12:50 – 1:30

CDRH Portal: Overview and Feature Walkthrough

The CDRH portal is a new platform that provides industry with secure submission progress tracking and online upload for CDRH premarket submissions. This presentation will provide an overview of the CDRH Portal and its current features.

Nelson Anderson, B.S.
Platform Owner, CDRH Portal
 Division of Regulatory Systems, Tools, and
 Data Management
 Office of Regulatory Programs (ORP)
 Office of Product Evaluation and Quality
 (OPEQ) | CDRH

1:30 - 2:10

Reduced Medical Device User Fees: Small Business Determination (SBD) Program

Do you want to save money on your CDRH marketing application fee? Are you a Small Business with gross receipts and sales of less than \$100 million for the most recent tax year? If so, you could be eligible for a Small Business Determination (SBD) and reduced or waived User Fee for your submission. This session discusses the SBD qualification requirements, essential definitions, and submission process. It will conclude with helpful tips and strategies to avoid common mistakes in SBD Certification Requests that might delay your approval.

Jason Brookbank
Assistant Division Director
 Division of Financial Management
 Office of Management (OM) | CDRH

Suggested pre-requisite:

- [Reduced Medical Device User Fees: Small Business Determination \(SBD\) Program](#)
- [How to Complete Form FDA 3602: MDUFA Small Business Qualification and Certification for a Business Headquartered in the United States](#)
- [How to Complete Form FDA 3602A: MDUFA Foreign Small Business Qualification and Certification Request for a Business Headquartered Outside the United States](#)

2:10 - 2:30 PM: BREAK

DAY THREE: CDRH Sessions: Wednesday, June 7, 2023

2:30 – 2:40

Welcome to REdl 2023 Device Track, Part 2

Joseph Tartal
Deputy Director
 DICE | OCE | CDRH

2:40 – 3:20

Managing Medical Device Nonconforming Product with Quality

Manufacturers are required to establish and maintain procedures to control product that does not conform to specified requirements. But what do you do when you have nonconformances? Do you continue to market nonconforming products, rework nonconforming products before marketing or do you dispose of the nonconforming products? This presentation will answer these questions and compare the requirements of the Quality System Regulation, 21 CFR 820.90, and ISO 13485:2016.

Ruth Bediakoh
Consumer Safety Officer
 Postmarket and Consumer Branch
 DICE | OCE | CDRH

3:20 – 4:00

Handling Medical Device Complaint Files with Quality

Manufacturers are required to maintain complaint files and procedures for handling medical device complaints. These procedures can vary a great deal depending on factors including risk, size of the company and complexity of the device. This presentation will provide the regulatory requirements for medical device complaint files. It will also provide key components of complaint files, common pitfalls and challenges associated with managing complaint files, and review strategies for addressing them.

Tonya A. Wilbon
Branch Chief
 Postmarket and Consumer Branch
 DICE | OCE | CDRH

4:00 – 4:05

CDRH Day One Closing Remarks

Joseph Tartal
Deputy Director
 DICE | OCE | CDRH

4:05 PM: DAY THREE ADJOURN

DAY FOUR: CDRH Sessions: Thursday, June 8, 2023

8:30 – 8:45

CDRH Day Two Welcome & Overview

Joseph Tartal
Deputy Director
DICE | OCE | CDRH

8:45 – 9:25

Addressing Regulatory Science Gaps in Artificial Intelligence (AI) and Machine Learning (ML)

Artificial intelligence (AI)/machine learning (ML)-based technologies have the potential to transform healthcare. However, due to the rapid application of the technology into many different areas and types of medical data, there are many challenges in developing robust evaluation methods, and in better understanding the effects of these devices in the real world. The AI/ML research program at CDRH's Office of Science and Engineering Laboratories (OSEL) focuses on test methodologies for assessing AI/ML performance both in the pre-market and real-world settings to reasonably ensure the safety and effectiveness of novel AI/ML algorithms. This presentation will cover the regulatory science gaps pertaining to the medical AI/ML space, and efforts by OSEL researchers to address these gaps.

Alexej Gossmann, PhD
Staff Fellow
Division of Imaging, Diagnostics, and Software
Reliability (DIDSR)
Office of Science and Engineering Laboratories
(OSEL) | CDRH

9:25 – 10:05

Radiation-Emitting Medical Devices Update

The FDA issued a final rule in February 2023, to amend and appeal certain radiological health regulations that will clarify and update these regulations to reduce the burden of regulatory requirements for radiation emitting products and medical devices without compromising patient safety. This presentation provides an overview of key amendments to the electronic product radiation control (EPRC) regulations. It will focus on those changes to the EPRC regulatory requirements for some electronic radiation emitting products which are also medical devices (such as diagnostic x-ray medical equipment), and medical devices which incorporate radiation emitting products into their final assembly (such as medical devices which incorporate a laser component).

Laurel Burk, PhD
Director
Division of Radiological Imaging Devices and
Electronic Products
Office of Radiological Health (Office of Health
Technology 8)
Office of Product Evaluation and Quality (OPEQ)
CDRH

Suggested pre-requisite:

- [How To Get Your Electronic Product on the US Market](#)
- [Electronic Product Certification and Quality Testing Programs](#)

10:05 - 10:25: BREAK

DAY FOUR: CDRH Sessions: Thursday, June 8, 2023

10:25 – 11:05

CDRH Medical Device Import Overview

The Food and Drug Administration (FDA) is responsible for ensuring that medical devices (including in vitro diagnostics and radiation-emitting products) comply with applicable United States (U.S.) regulations at every point of the device cycle, to include those of foreign origin. Foreign establishments must comply with these applicable regulations before, during, and after the medical device is imported into the U.S. or territory. This presentation will provide a brief overview of the import process of medical devices. It will highlight many aspects of the import cycle, answer frequently asked questions, and provide the audience with links to resources regarding the importation process.

Yvette Montes

Consumer Safety Officer
Imports and Registration and Listing Team (IRLT)
Division of Regulatory Programs 2 (DRP2)
Office of Regulatory Programs (ORP)
Office of Product Evaluation and Quality (OPEQ)
CDRH

11:05 – 11:45

All About the Form FDA 483 and ORA Electronic Reading Room

After an inspection, your firm might be issued a Form FDA 483 (483). This presentation will provide an overview of what it means to get a 483, your firm's responsibility, and how to respond to the FDA. This presentation will also include an overview of the ORA Freedom of Information Act (FOIA) Electronic Reading Room, which is a public site that displays copies of ORA domestic inspection and related records.

William Chang, MBA, PE

Lieutenant Commander, US Public Health Service
Office of Medical Device and Radiological Health
Operations (OMDRHO)
Division 1 - East
Office of Regulatory Affairs (ORA)

Suggested pre-requisites:

- [Office of Regulatory Affairs](#)

11:45 – 11:55

Closing for CDRH Sessions

Joseph Tartal

Deputy Director
DICE | OCE | CDRH

11:55 – 1:00: LUNCH BREAK

1:00 – 1:05

Welcome Back

DAY FOUR: CBER Sessions: Thursday, June 8, 2023

AGENDA

All times are Eastern (EDT UTC-4)

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June 5-6

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June 8-9

1:05 – 1:15

CBER Sessions Welcome

Larissa Lapteva, MD, MHS, MBA

Associate Director

Division of Clinical Evaluation General Medicine (DCEGM)

Office of Clinical Evaluation (OCE)

Office of Therapeutic Products (OTP)

Center for Biologics Evaluation and Research (CBER)

1:15 – 2:05

PDUFA VII Enhancements- Interactions with Office of Therapeutic Products (OTP)

This presentation will provide an overview of new and enhanced programs for interactions with OTP as part of the Prescription Drug User Fee Act (PDUFA) VII.

Includes Question and Answer Session

Mara Miller, MA

Division Director

Division of Review Management and Regulatory Review 2

Office of Review Management and Regulatory Review

OTP | CBER

2:05 – 3:00

Overview of Pediatric Research Equity Act (PREA) and Rare Pediatric Disease PRVs

This presentation will discuss the Pediatric Research Equity Act (PREA), Pediatric Study Plans (PSPs) and the Rare Pediatric Disease Priority Review Voucher (RPD PRV) program with a focus on CBER-regulated products.

Includes Question and Answer Session

Adrienne Hornatko-Munoz, RAC-US

Senior Project Manager

Office of the Regulatory Operations (ORO)

CBER

3:00 – 3:05: BREAK

DAY FOUR: CBER Sessions: Thursday, June 8, 2023

3:05 – 4:35 Early Stages of Product Development: What You Need to Know

3:05 – 3:25

Preclinical Development for Cellular and Gene Therapy Products

In this presentation early interaction opportunities with CBER, including INTERACTs and pre-INDs meetings, will be explained and discussed from the perspective of preclinical product development.

Ernesto Moreira, MD
Biologist
 Pharmacology/Toxicology Branch I
 OTP | CBER

3:25 – 3:45

Preclinical Considerations for the Development of Cellular and Gene Therapy Products for IND Submissions

This presentation will provide information regarding the pharmacology and toxicology part of IND applications, common deficiencies observed in INDs, and the concept of prospect of direct benefit as it applies to preclinical development of OTP-regulated products.

Gregory Conway, PhD, MA
Biological Reviewer
 Office of Pharmacology and Toxicology (OPT)
 OTP | CBER

3:45 – 4:15

Clinical Readiness for IND Submissions

This presentation will focus on clinical considerations for first-in-human (FIH) study design in cell, gene, and tissue-based products, including considerations for rare diseases.

Shelby Elenburg, MD
Medical Officer
 Division of Clinical Evaluation General Medicine (DCEGM)
 OCE | OTP | CBER

4:15 – 4:35

Questions & Answers

**Ernesto Moreira, Gregory Conway,
 Shelby Elenburg**

4:35 – 4:40

CBER Day One Closing Remarks

Larissa Lapteva, MD, MHS, MBA
Associate Director
 DCEGM | OCE | OTP | CBER

4:40 PM: DAY FOUR ADJOURN

DAY FIVE: CBER Sessions: Friday, June 9, 2023

8:30 – 8:35

CBER Day Two Welcome & Overview

Larissa Lapteva, MD, MHS, MBA
Associate Director
 DCEGM | OCE | OTP | CBER

8:35 – 9:30

CMC Developmental Readiness Pilot (CDRP) Program

This presentation will discuss Accelerating Product Development and the FDA’s PDUFA VII initiative to help Cell and Gene and other Advanced Therapy Sponsors meet Chemistry Manufacturing and Controls (CMC) Regulatory Expectations in BLAs through a CMC Developmental Readiness Pilot (CDRP) Program.

Ramjay Vatsan, PhD, CQA
Associate Director for Policy
 Office of Gene Therapy
 OTP | CBER

Includes Question and Answer Session

9:30 – 9:35: BREAK

9:35 – 10:30

CMC Considerations for Tissue Engineered Product Development

This presentation will discuss the CMC requirements for tissue engineered products, including considerations for early phase product development and product characterization.

Wen (Aaron) Seeto, PhD
Staff Fellow
 Tissue Engineering Branch 2
 Division of Cell Therapy 2
 Office of Cellular Therapy & Human Tissue CMC
 (OCTHT) | OTP | CBER

Includes Question and Answer Session

10:30 – 10:35: BREAK

10:35 – 11:30

Identifying and Controlling Attributes Related to Potency for Cell and Gene Therapy Products

This presentation will discuss approaches to identifying product attributes related to potency for cell and gene therapy products and developing meaningful potency assays for lot release testing and comparability assessments.

Matthew Klinker, PhD
Biologist/CMC Reviewer
 Cell Therapy Branch 2 (CTB2)
 OCTHT | OTP | CBER

Includes Question and Answer Session

11:30 – 12:30: LUNCH BREAK

12:30 – 12:35

Welcome Back

DAY FIVE: CBER Sessions: Friday, June 9, 2023

12:35 – 1:30

Overview and Updates on FDA’s Implementation of the Estimand Framework and Complex Innovative Trial Design Review Program

This presentation will provide an introduction and recent updates on two FDA initiatives related to modernizing clinical trial design: The estimand framework of ICH E9(R1) and the PDUFA VI / PDUFA VII complex innovative trial design (CID) paired meeting program.

Includes Question and Answer Session

John Scott, PhD, AM
Director
 Division of Biostatistics (DB)
 Office of Biostatistics and Pharmacovigilance (OBPV)
 | CBER

1:30 – 1:35: BREAK

1:35 – 2:30

Postmarketing Safety and Pharmacovigilance for Vaccines

This presentation will provide an overview of postmarketing safety monitoring for vaccines, including examples of pharmacovigilance activities, such as safety-related postmarketing studies.

Includes Question and Answer Session

Meghna Alimchandani, MD
Deputy Director
 Division of Pharmacovigilance (DPV)
 OBPV | CBER

2:30 – 2:40: BREAK

2:40 – 3:35

Expanded Access to Investigational Biologics for Treatment Use

This presentation will outline the regulatory requirements on expanded access to investigational drugs/biologics for treatment use and will provide practical recommendations on how to submit expanded access IND requests to FDA/CBER.

Includes Question and Answer Session

Lei Xu, MD, PhD
Branch Chief
 General Medicine Branch 2 (GMB2)
 DCEGM | OCE | OTP | CBER

3:35 – 3:40: BREAK

DAY FIVE: CBER Sessions: Friday, June 9, 2023

3:40 – 4:30

Requirements and GMP Inspection of Facility for Cell and Gene Therapy Products

This presentation will summarize the systems that are covered by the FDA pre-license inspection (PLI) and pre-approval inspection (PAI) of facilities for cell and gene therapy products, including (but are not limited to) the facility design and qualifications, quality systems, and examples of objectionable observations.

Includes Question and Answer Session

Wei Wang, PhD

Microbiologist

Division of Manufacturing and Product Quality (DMPQ)

Office of Compliance and Biologics Quality (OCBQ)

CBER

4:30 – 4:35

CBER & Conference Closing Remarks

Larissa Lapteva, MD, MHS, MBA

Associate Director

DCEGM | OCE | OTP | CBER

4:35 PM: CONFERENCE ADJOURN